Part I: Introduction to Biostatistics
Resources at NYULH

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June 16, 2022,
Where is NYUBR?

- **Department of Population Health**
  - Chair: Marc N. Gourevitch, MD, MPH

- **Division of Biostatistics**
  - Director: Andrea B. Troxel, ScD

- **NYU Biostatistics Resource**
  - Director: Huilin Li, PhD
  - Associate Director: Jiyuan Hu, PhD

Location: 180 Madison Ave, the second, fourth and fifth floors

[Biostatistics-resource@nyulangone.org](mailto:Biostatistics-resource@nyulangone.org)
NYUBR Research Team

- 7 Professors
- 6 Associate Professors
- 9 Assist. Professors

Faculty

- 6 PhDs
- 16 Masters

Research Scientists

- 6 Postdocs

Postdoctoral Fellows

Admin Staff
Our Expertise:

- Study design
- Advanced analytical methods
- Data (clinical) coordinating center creation and implementation
- Core leadership in center grants
Our Expertise: Study Design

“TO CONSULT THE STATISTICIAN AFTER AN EXPERIMENT IS FINISHED IS OFTEN MERELY TO ASK HIM TO CONDUCT A POST MORTEM EXAMINATION. HE CAN PERHAPS SAY WHAT THE EXPERIMENT DIED OF.”

Ronald A. Fisher (1890 – 1962)
Our Expertise: Study Design

Reproducible Research Needs Good Design!
Our Expertise: Study Design

**Observational**
- Cohort
- Case-control
- Complex survey
- EHR

**Experimental**
- Clinical Trials
- Laboratory
- High throughput
  - Standard
  - Adaptive
  - Pragmatic
  - Multifactor
  - Longitudinal
Our Expertise: Advanced Analytical Methods

Hierarchical model fitting

Survival analysis

Longitudinal analysis

Network analysis

Clustering, classification, and prediction

High-dimensional omics data analysis
Types of Biostatistics Collaborations

Short-term support
- developing pilot study designs
- performing statistical analysis on a prepared dataset
- preparing abstracts or other presentations
- preparing manuscripts (either initial or for a revision)

Grant proposals
- assisting in refining study questions and measurement methods
- developing study and experimental designs
- writing statistical analysis plans
- computing precision, power, and sample sizes

Long-term support
- proposal development
- manuscript writing
- statistical analysis
- presence at departmental research meetings
- participation in journal clubs (methodological review)
- assistance with research conference presentations, and K-award mentoring
Core Resources

NYUBR

- BERD at Clinical and Translational Science Institute (CTSI)
- The Biostatistics Shared Resources at Perlmutter Cancer Center
- Environmental Health Statistics and Bioinformatics Facility Core (EHSB)
- Alzheimer’s Disease Center’s Data Management and Statistics Core
- Other cores
Biostatistics, Epidemiology and Research Design Program (BERD) in NYU-H+H Clinical & Translational Science Institute (CTSI)

Translational research transforms basic science discoveries into practical applications that can improve health, such as new diagnostic techniques, therapies, and interventions.

BERD provides biostatistical, epidemiological, and study design collaborations for projects with translational research component

- Consultations
- Grant developments and data analyses (vouchers can be applied)
- Studios (multidisciplinary project reviews)
- IRB scientific reviews
- Trainings
**Biostatistics Tool**
Enable investigators independently implement relatively straightforward tasks:
- Power calculators
- Sample size calculators
- Data analysis calculators (t-test, correlations, linear regressions, ROCs etc.)

**K Application Resource Repository**
Enable efficient mentoring with shared resources on:
- successful grant writing and summary statements examples
- career development templates and examples
- biostatistics resources and training catalogs
- letter of support templates

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**Biostatistics Tools**

The Biostatistics, Epidemiology, and Research Design team at NYU Langone’s Clinical and Translational Science Institute have compiled useful biostatistics tools for statistical analyses.

The tools include calculators for study design, such as power and sample size calculation, and calculators for data analysis such as t-test, chi-square test, Pearson correlation coefficient, linear regression, and receiver operating characteristic (ROC) curve. If you need additional research advice, please submit our online form to request a consultation.

**Tools for Study Design**

Several online tools are available for researchers to calculate statistical powers or sample sizes during the study design phase.

**Power Calculator**

When a study sample size is determined, researchers may wish to calculate the statistical power of the statistical hypothesis for the given study sample size. Inputs typically contain sample size (n), the effect size, the number of groups, and the desired type I error rate (the probability of rejecting the null hypothesis when it is true, typically set at 0.05 when there is no multiple testing corrections), and one-sided or two-sided test.

For continuous outcomes, use the two-sample unequal t-test power calculator to test the hypothesis that the outcome has the same mean between two groups or the analysis of variance (ANOVA) power calculator to test to test the hypothesis that the outcome has the same mean between multiple groups.

For categorical outcomes, use the chi-squared test power calculator to test the hypothesis that the probability of outcome is the same across groups. For continuous outcome and continuous exposure, use the linear regression power calculator to test the hypothesis that the outcome is independent of the exposure, with or without covariates adjusted.

**Sample Size Calculator**
Biostatistics Shared Resource—Perlmutter Comprehensive Cancer Center (PCC)

To provide state-of-the-art statistical collaboration and consultation to members of the Perlmutter Cancer Center (PCC) basic science, clinical and translational research programs and Shared Resources.

BSR provides biostatistical, epidemiological, and study design collaborations provided by dedicated cancer-focused faculty and staff of Biostatistics.

1. Develop new peer-reviewed **grant applications**
2. Develop research designs and **protocols for investigator-initiated interventional treatment clinical trials**
3. Provide ongoing statistical collaboration for **PRMC-approved, PCC investigator-initiated interventional treatment clinical trials**
4. Develop research designs and protocols for new PRMC-approved, investigator initiated clinical trials, **non-interventional, and observational studies**
5. **Training and mentoring of PCC members and staff** in key statistical issues for study design, analysis, and interpretation of cancer research studies
NYUBR Collaboration Process

Division of Biostatistics website

Scientific Cores & Shared Resources—Biostatistics Resource website

Inquiry Form

NYUBR

CTSI  Cancer Center  EHSB  Alzheimer Core  Others

Contact us: Biostatistics-resource@nyulangone.org

Designated faculty and staff
Thank you

NYU Biostatistics Resource email: Biostatistics-resource@nyulangone.org

Division of Biostatistics website: https://med.nyu.edu/departments-institutes/population-health/divisions-sections-centers/biostatistics/research

Scientific Cores & Shared Resources—Biostatistics Resource website: https://med.nyu.edu/research/scientific-cores-shared-resources/biostatistics-resource
Part II: Tips for Preparing Grant Proposals from Statistical Reviewers

Panelists: Drs. Andrea B. Troxel, Huilin Li, Judy Zhong, Mengling Liu, Erinn M. Hade

Moderated by Dr. Jiyuan Hu

June 16, 2022,
Perspectives from a Statistical Reviewer in Study Sections and Grant Review Panels

• Study sections often have a statistical reviewer for applications involving human subjects and basic science projects involving *omics* analysis

• Statistical review emphases:
  • **Significance**
    • Is the proposed hypothesis statistically testable?
  • **Investigators**
    • Does the team have statistical expertise to design, monitor and analyze?
    • Are the budgeted efforts sufficient to implement the scope of work?
  • **Innovation**
    • Innovative design of the trial and analysis of the observational data is especially appreciated
  • **Approach**
    • Is the proposed strategy likely to produce unbiased and interpretable results?
    • Does the study provide adequate power, use an appropriate study population?
    • Does the application appropriately account for sex and relevant biological variables, and potential confounders?
General Tips

• **Initial contact**
  - Translate clearly defined scientific questions into statistical hypotheses
  - Ensure subject enrollment and sample collection address scientific question and enable data analysis

• **Study design**
  - Driven by scientific questions: interventional vs. observational, retrospective vs. prospective, targeted vs. untargeted, fixed vs. adaptive, controlled vs. pragmatic.
  - Determined by projected effect sizes: preliminary studies or literature
  - Constrained by resource: accessibility to study population, funding, compliance factors

• **Preliminary results**
  - Generate hypothesis and provide rationale of the proposed investigation
  - Provide supporting information to plan the project

• **Sample Size and Power**
  - Sample size often determined for primary outcome; show sufficiency for secondary outcomes
  - Projected effect size: clinical meaningful and well supported by preliminary results or literature
  - Account for multiple testing and potential missing
  - Simple models and assumptions for power analysis

• **Statistical analysis plan**
  - Address the scientific questions: clarity is key with proper approaches (not over-complicated)
  - Cover potential questions: missing data, adjusting confounders, nonlinear effects, dimensional reduction

• **Potential pitfalls and solutions**
Specific Tips for Large P or Center Grant

• Longer preparation time and larger team
  • Multiple faculty biostatisticians to provide sufficient expertise and supports

• Biostatistics core
  • Coordinate with the other administrative cores
  • Collaborate with all research projects

• Statistical plans
  • Summarize core activities across the entire the core document
  • Specific analysis plans for each research project
  • Show synergy/interactions across projects and cores
Specific Tips for Clinical Trial Grant

• Clinical Trial
  • Justification for choice of design
  • Discussion of randomization/masking
  • Clear statement of primary/secondary/exploratory objectives
  • Consideration of interim analyses, monitoring, and DSMB

• Data & Safety Monitoring Board
  • General consideration
  • Align Board expertise with trial goals
  • Budget considerations
  • CTSI resource
Specific Tips for Other Types of Research Grant

• **Observational Study**
  • Validation cohort is important
  • Ancillary study of the ongoing cohorts
  • Secondary analysis of existing study

• **Molecular Mechanism Study**
  • High dimensional data—multiple testing adjustment
  • Association vs. Prediction
  • Analysis approaches to reflect the updates in modern technology
  • Batch effect
  • Streamlined upstream and downstream data analysis
  • Integrated analysis taking account of study design, omics data, and clinical outcomes
Role of Statisticians by Grant Mechanisms

- **Large P or Center Grant** – Core Director, Co-I, or statistical analyst
- **Two-phase grant UG3/UH3** – MPI, Co-I, or statistical analyst
- **Large research grant: R01/U01** – MPI, Co-I, or statistical analyst
- **Small research grant: R03/R21** – Co-I
- **Training grant** – Mentor
Case Study with the Suggested Timeline

8 weeks
Contact statistician

6-8 weeks
Introduce background, exchange literature

4-6 weeks
Review/refine study design, hypothesis, preliminary analysis

2-4 weeks
Finalize the study design and sample size

1-2 weeks
Finalize the statistical analysis plan

0 week
Submission
Thank you

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