RESERACH METHODS FOR CLINICAL INVESTIGATORS
Session 5:

Calculating Sample Size and Statistical Power for a Study

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Objectives

At the end of the presentation, the audience will be able to:

- Understand the importance of statistical power in a study
  - Hypothesis Testing
- Determine the sample size of a study
- Determine the statistical analysis plan after calculating sample size
- Summarize the components needed to apply for NIH funding

Power & Sample Size

Why are they important in research?

- Includes a small sample of subjects w/ a particular characteristic vs. the whole population→ Make inferences for the whole population

- Determine the number of subjects needed in a study

  -Helps to answer the hypothesis

Jones, SR. (2004). An Introduction to power and sample size estimation. *Emergency Medicine Journal*. http://dx.doi.org/10.1136/emj.20.5.453

Hypothesis Testing

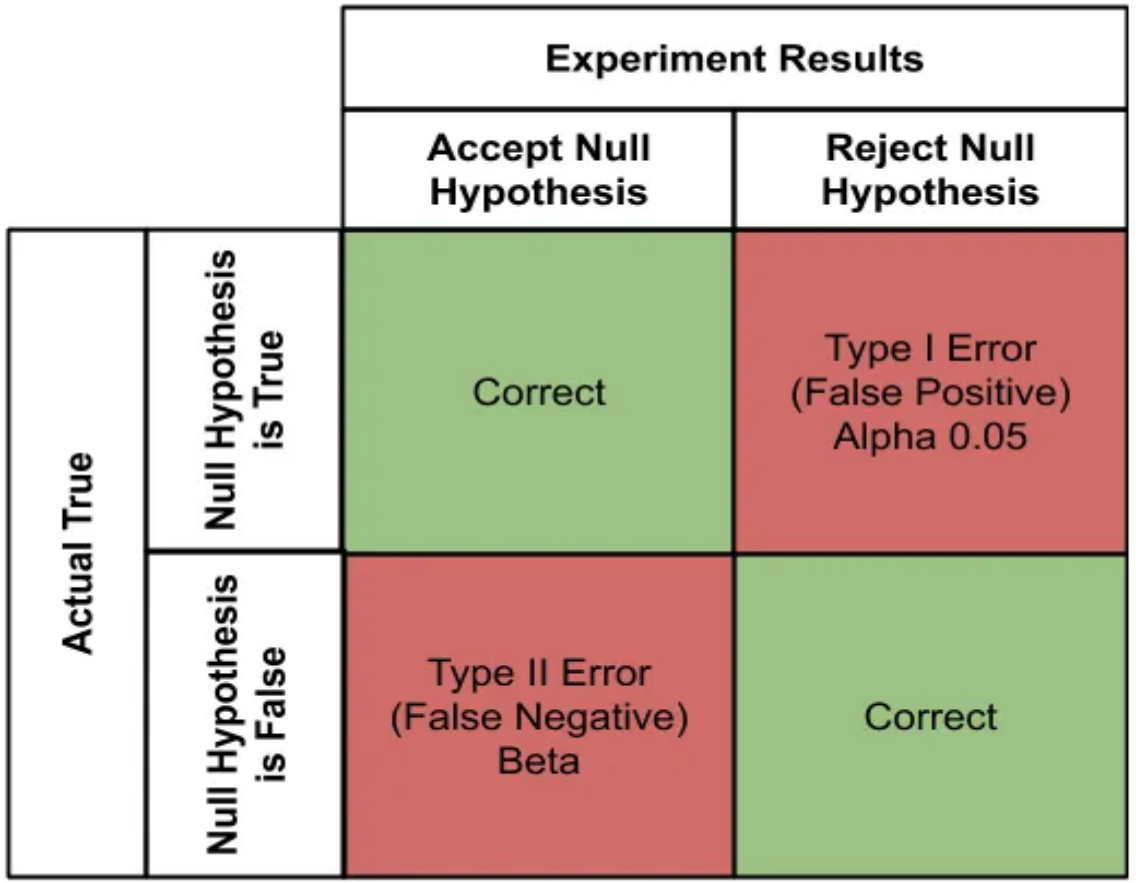
Statistical procedure used to make an assumption about the parameters of a population or its distribution

Null (H_0): Null hypothesis states that there is NO statistical relationship

Alternative (H_A): Alternative hypothesis states that there is a statistical relationship

Motulsky, Harvey. (2014). *Intuitive Biostatistics: Nonmathematical Guide to Statistical Thinking*. Oxford University Press

Hypothesis Testing cont'd



1.) Gordis, Leon. (2018). *Epidemiology*. Saunders Elsevier; 2.) Schmidt, S. (2018). Research Techniques Made Simple: Sample Size Estimation and Power Calculation. Journal of Investigative Dermatology. Vol 138(8). https://doi.org/10.1016/j.jid.2018.06.165

Hypothesis Testing cont'd

- Type I Error (False Positive)
  - Erroneous conclusion that study results between groups **<u>are</u>** different when in fact they really **<u>aren't</u>** different
  - Reject the Null (H_0) when in fact the Null (H_0) is ***<u>TRUE</u>***
- Probability of Type I Error: α (alpha)

1. Motulsky, Harvey. (2014). *Intuitive Biostatistics: Nonmathematical Guide to Statistical Thinking*. Oxford University Press
2. Gordis, Leon. (2018). *Epidemiology*. Saunders Elsevier.

Hypothesis Testing cont'd

- Type II Error (False Negative)
  - Erroneous conclusion that study results between groups **<u>aren't</u>** different when in fact they really **<u>are</u>** different
  - Fail to reject the **Null (H_0) when in fact the Null (H_0) is *<u>FALSE</u>***
- Probability of Type II Error: β (Beta)

1. Motulsky, Harvey. (2014). *Intuitive Biostatistics: Nonmathematical Guide to Statistical Thinking*. Oxford University Press
2. Gordis, Leon. (2018). *Epidemiology*. Saunders Elsevier.

Hypothesis Testing cont'd

- Probability of Type I Error
  - α (alpha) → p-value, i.e. $p < 0.05$

- Probability that such a difference could have arisen by chance

Caveat: Type I Error should be small thus illustrating very little error in the study when making conclusions from results

Gordis, Leon. (2018). *Epidemiology*. Saunders Elsevier.

Hypothesis Testing cont'd

- Probability of Type II Error
  - β (Beta) → Power of study (1- β)

- Probability that it is correctly determined that the study results between groups DIFFER

Gordis, Leon. (2018). *Epidemiology*. Saunders Elsevier.

Type I & Type II Error

	Decision: Delete as Junk	Decision: Place in Inbox
Good Email	Type I Error	(No Error)
Spam	(No Error)	Type II Error

Motulsky, Harvey. (2014). *Intuitive Biostatistics: Nonmathematical Guide to Statistical Thinking*. Oxford University Press

Sample Size Calculations

Study type	Formulas	Explanations
Proportion in survey type of studies	$N = \frac{Z^2_{\alpha/2} \times P \times (1 - p) \times D}{E^2}$	**N** - sample size **P** - prevalence or proportion of event **E** - precision (or margin of error) with which a researcher want to measure something **D** - design effect reflects the sampling design used in the survey type of study. This is 1 for simple random sampling and higher values (usually 1 to 2) for other designs such as stratified, systematic, cluster random sampling $\mathbf{Z}_{\alpha/2}$ - 1.96 for alpha 0.05

Serdar, C. (2021). Sample size, power and effect size revisited: simplified and practical approaches in pre-clinical, clinical and laboratory studies. *Biochem Med (Zagreb)*. Vol 31(1). doi: 10.11613/BM.2021.010502

Sample Size Calculations cont'd

Group mean

$$N = Z^2_{\alpha/2}\, s^2/d^2$$

s - standard deviation obtained from previous study, or pilot study

d - accuracy of estimate or how close to the true mean

$Z_{\alpha/2}$ -1.96 for alpha 0.05

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Statistical Analysis Plan

1. Method of Data Collection (Primary vs. Secondary)
   a) Survey: Questionnaire
   b) Medical Charts
   c) Interviews

2. Hypothesis Testing: Reject/Accept (Null=H_0 vs. Alternate=H_A)

   p-value > 0.05= Accept the H_0 and reject H_A, No difference in groups

   p-value ≤ 0.05= Reject the H_0 and accept H_A, Difference in groups

1. Dahiru.T., et al. (2008). P-value, A true test of statistical significance; A cautionary note. *Ann Ib Postgrad Med*. 6(1): 21-26. doi: 10.4314/aipm.v6i1.64038
2. Gordis, Leon. (2018). *Epidemiology*. Saunders Elsevier

Statistical Analysis Plan cont'd

3. Measures of Association

- Odds Ratio (OR): Case-Control
- Relative Risks (RR): Cohort
- Hazard Ratio (HR): Randomized Clinical Trials

Research Question → Study Design → Data Collection → Analysis → Publication

Applying for NIH Funding

Necessary:

1. Sample size estimation

2. Study Power

***Hypothesis testing and generalizability to whole population

3. Statistical Analysis Plan

Applying for NIH Funding cont'd

Necessary:

Statistical Analysis Plan

**Must analyze data by gender and race regardless of detecting statistical differences between groups

https://www.researchmethodsresources.nih.gov/
https://www.prevention.nih.gov/education-training/pragmatic-and-group-randomized-trials-public-health-and-medicine

Summary

Specifications of Estimating Sample Size

1. Difference in response rates to be detected
2. Estimate of the response rate in one of the study groups
3. Level of statistical significance (α)
4. Value of power desired ($1-\beta$)
5. Hypothesis Testing: 1-sided vs. 2-sided tests

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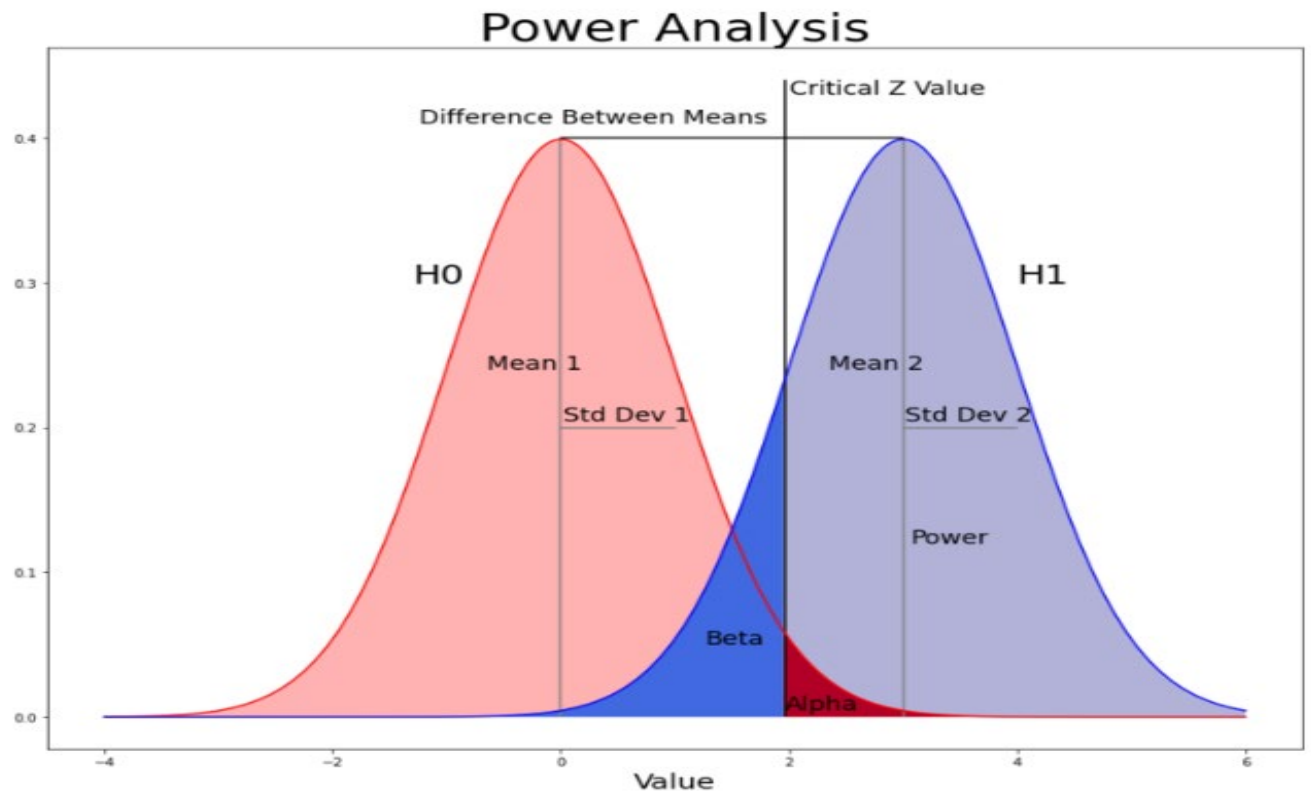
Summary

Power Analysis Statistics



Armstrong, A. (2022). How many Samples Do I Need? Determining Sample Size for Statistically Significant Results. *The BYU Design Review*