

Sample size determination and Power of a study

Sample Size Determination for Simple Comparative Experiments

Overview:

- In clinical studies that compare two or more groups (e.g., treatment vs. control), the **sample size** is the number of participants required in each group to detect a **statistically significant difference** if one truly exists.
- Getting the sample size right is **critical**—too small, and you may miss a real effect; too large, and you may waste resources or expose more participants than needed to risk.

Purpose of Sample Size Calculation:

- To **ensure adequate statistical power** (usually 80–90%)
- To **detect a clinically meaningful difference** (effect size)
- To **minimize Type I (false positive) and Type II (false negative) errors**
- To avoid **over- or under-recruitment**, which can lead to ethical and financial issues

Core Elements Required:

1. Significance Level (α):

- Usually set at 0.05 (5%)
- This is the probability of making a **Type I error**, i.e., rejecting a true null hypothesis

2. Power ($1 - \beta$):

- Commonly 0.80 (80%) or 0.90 (90%)
- This is the probability of **correctly rejecting the null hypothesis** when a real effect exists

3. Effect Size:

- The **magnitude of the difference** expected between groups (e.g., 5 mmHg drop in blood pressure)
- Larger effect sizes require smaller sample sizes; smaller effect sizes need larger samples

4. Variability (Standard Deviation):

- The more variable the data, the larger the sample size needed to detect a given effect
- Estimated from previous studies or pilot data

5. Type of test:

- Two-sided vs. one-sided

- Type of outcome (continuous, binary, or time-to-event)

Example (Continuous Outcome, Two Groups):

- For comparing **means** between two independent groups (e.g., drug vs. placebo), a typical formula:

$$n = \frac{2(Z_{\alpha/2} + Z_{\beta})^2 \cdot \sigma^2}{\Delta^2}$$

- Where:
 - n = sample size per group
 - $Z_{\alpha/2}$ = Z-value for chosen significance level (e.g., 1.96 for 0.05)
 - Z_{β} = Z-value for power (e.g., 0.84 for 80% power)
 - σ = standard deviation of the outcome
 - Δ = minimum clinically important difference (effect size)

Example:

- Suppose you want to compare the effect of two diets on systolic blood pressure.
 - Expected standard deviation (σ) = 10 mmHg
 - Clinically important difference (Δ) = 5 mmHg
 - Significance level (α) = 0.05 $\rightarrow Z_{\alpha/2} = 1.96$
 - Power = 80% $\rightarrow Z_{\beta} = 0.84$

$$n = \frac{2 \cdot (1.96 + 0.84)^2 \cdot 10^2}{5^2} = \frac{2 \cdot (2.8)^2 \cdot 100}{25} = \frac{2 \cdot 7.84 \cdot 100}{25} = \frac{1568}{25} = 62.72$$

- Answer:**
 - You need **63 participants per group**.

Software Tools:

- G*Power
- OpenEpi
- nQuery
- PASS
- R (power.t.test, pwr package)

Determination of Sample Size to Obtain a Confidence Interval of Specified Width

Overview:

- This calculation is not for comparing groups, but for estimating a single parameter (like a mean or proportion) with a specified level of precision.
- The goal is to determine a sample size such that the confidence interval (CI) around your estimate is no wider than a specified margin.

Why This Is Important:

- Used in prevalence studies, surveys, descriptive epidemiology, and early-phase research
- You may want to estimate the average value or proportion with narrow uncertainty

Key Concepts:

1. Confidence Level (typically 95%):

- This gives you a **range** around your estimate that is likely to contain the true population value
- Corresponds to a **Z-score** (e.g., 1.96 for 95% CI)

2. Margin of Error (E):

- The **half-width** of the desired confidence interval
- Smaller margin → more precision → larger sample size

3. Population Standard Deviation (σ) for estimating a mean

- Or estimated proportion (p) for estimating a percentage

Formulas:

A. Estimating a Population Mean:

$$n = \left(\frac{Z_{\alpha/2} \cdot \sigma}{E} \right)^2$$

- Where:
 - $Z_{\alpha/2}$ = Z-value for desired confidence (e.g., 1.96 for 95%)
 - σ = population standard deviation
 - E = desired margin of error (half the CI width)

Example (Mean):

- You want to estimate the average cholesterol level with:
 - Standard deviation (σ) = 15 mg/dL
 - Desired margin of error (E) = 3 mg/dL
 - 95% confidence → $Z_{\alpha/2} = 1.96$

$$n = \left(\frac{1.96 \cdot 15}{3} \right)^2 = \left(\frac{29.4}{3} \right)^2 = (9.8)^2 = 96.04$$

- **Answer:** At least **96 participants** are needed.

B. Estimating a Proportion (e.g., prevalence):

$$n = \frac{Z_{\alpha/2}^2 \cdot p(1-p)}{E^2}$$

➤ Where:

- p = expected proportion
- E = desired margin of error

Example (Proportion):

- You want to estimate the prevalence of anemia:
 - Expected prevalence = 20% → $p = 0.2$
 - Desired margin of error = ±5% → $E = 0.05$
 - Confidence level = 95% → $Z_{\alpha/2} = 1.96$

$$n = \frac{1.96^2 \cdot 0.2 \cdot 0.8}{0.05^2} = \frac{3.8416 \cdot 0.16}{0.0025} = \frac{0.6147}{0.0025} = 245.88$$

- **Answer:** At least **246 participants** are needed.

Power of a Study

Definition:

- The power of a study is the probability that it will correctly reject the null hypothesis when a true effect exists—i.e., it will **detect a difference** if there is one.
- Formally:

$$\text{Power} = 1 - \beta$$

- Where β is the probability of **Type II error**.

Why Is Power Important?

- Ensures the study is capable of detecting clinically meaningful differences
- Avoids wasting resources on underpowered studies (which may produce inconclusive results)
- Required by ethics committees and funding bodies
- **Low power** = high risk of **false negatives**



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