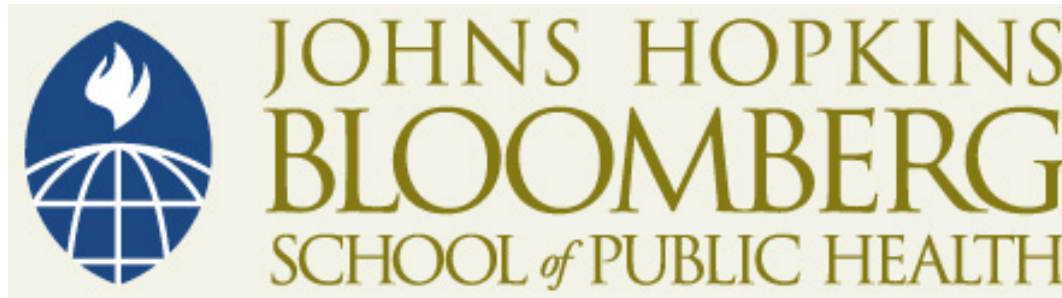


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Methods in Sample Surveys

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**Sample Size and Power
Estimation**

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Sample size and Power

“When statisticians are not making their lives producing confidence intervals and p-values, they are often producing power calculations”

Newson, 2001

”In planning of a sample survey, a stage is always reached at which a decision must be made about the size of the sample. The decision is important. **Too large** a sample implies a waste of resources, and **too small** a sample diminishes the utility of the results.“

Cochran, 1977

Sample size estimation: Why?

- Provides validity of the clinical trials/intervention studies – in fact any research study, even presidential election polls
- Assures that the intended study will have a desired power for correctly detecting a (clinically meaningful) difference of the study entity under study if such a difference truly exists

Sample size estimation

- ONLY two objectives:
 - Measure with a precision:
 - Precision analysis
 - Assure that the difference is correctly detected
 - Power analysis

First objective: measure with a precision

- Whenever we propose to estimate population parameters, such as, population mean, proportion, or total, we need to estimate with **a specified level of precision**
- **We like to specify a sample size that is sufficiently large to ensure a high probability that errors of estimation can be limited within desired limits**

Stated mathematically:

- we want a sample size to ensure that we can estimate a value, say, p from a sample which corresponds to the population parameter, P .
- Since we may not guarantee that p will be exact to P , we allow some error
- Error is limited to certain extent, that is this error should not exceed some specified limit, say d .

- We may express this as:

$$p - P = \pm d,$$

i.e., the difference between the estimated p and true P is not greater than d (allowable error: margin-of-error)

- But do we have any confidence that we can get a p , that is not far away from the error of $\pm d$?
- In other words, we want some confidence limits, say 95%, to our error estimate d .

That is $1-\alpha = 95\%$

It is a common practice: α -error = 5%

In probability terms, that is,

$$\mathit{prob} \{-d \leq p - P \leq d\} \geq 1 - \alpha$$

In English, we want our estimated proportion p to vary between $p-d$ to $p+d$, and we like to place our confidence that this will occur with a $1-\alpha$ probability.

From our basic statistical course, we know that we can construct a confidence interval for p by:

$$p \pm z_{1-\alpha/2}^* \text{se}(p)$$

where z_α denotes a value on the abscissa of a standard normal distribution (from an assumption that the sample elements are normally distributed) and $\text{se}(p) = \sigma_p$ is the standard error.

$$p \pm d = p \pm z_{1-\alpha/2} \sigma_p$$

Hence, we relate $p \pm d$ in probabilities such that:

$$\begin{aligned} d &= Z_{1-\alpha/2} \sigma \\ &= Z_{1-\alpha/2} \sqrt{\frac{p(1-p)}{n}} \end{aligned}$$

If we square both sides,

$$\begin{aligned}d &= Z_{1-\alpha/2} \sigma \\ &= Z_{1-\alpha/2} \sqrt{\frac{p(1-p)}{n}}\end{aligned}$$



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

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

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

For the above example:

$$n = \frac{(1.96)^2 * 0.4 * 0.6}{(.10)^2} = 92.2 \approx 93$$

Note that, the sample size requirement is highest when $p=0.5$. It is a common practice to take $p=0.5$ when no information is available about p for a conservative estimation of sample size.

As an example, $p = 0.5$, $d = 0.05$
(5% margin-of-error), and α -error = 0.05:

$$n = \frac{(1.96)^2 * 0.5 * 0.5}{(.05)^2} = 384.16 = 385 \approx 400$$

```
. di 1.96^2*.5*(1-.5)/(.05^2)
384.16
. di (invnorm(.05/2))^2*.5*(1-.5)/(.05^2)
384.14588
```

Stata

```
. sampsi .5 .55, p(.5) onsample
```

Estimated sample size for one-sample comparison of proportion
to hypothesized value

Test Ho: $p = 0.5000$, where p is the proportion in the population

Assumptions:

alpha = 0.0500 (two-sided)

power = 0.5000

alternative p = 0.5500

Estimated required sample size:

n = 385

Sample Size Estimation for Relative Differences

If d is relative difference,

$$n = \frac{t^2 p(1-p)}{(d * p)^2}$$

$$= \frac{t^2 (1-p)}{d^2 p}$$

Consider that 10% change is relative to $p=.40$ in the above example.

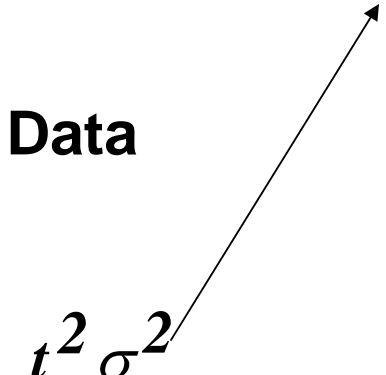
Then, $d = 0.4 * 0.10 = 0.04$, that is, p varies between 0.36 to 0.44. Now,

$$n = \frac{(1.96)^2 * 0.4 * 0.6}{(.10 * 0.4)^2} = 576.24 \approx 577$$

Note, d is very sensitive for sample size calculation.

Change the variance

Sample Size for Continuous Data

$$n = \frac{t^2 \sigma^2}{d^2}$$


Sources of variance information:

- Published studies
 - (Concerns: geographical, contextual, time issues – external validity)
- Previous studies
- Pilot studies

Study design and sample size

- Sample size estimation depends on the study design – as variance of an estimate depends on the study design
- The variance formula we just used is based on “simple random sampling” (SRS)
- In practice, SRS strategy is rarely used
- Be aware of the study design

Sample Size Under SRS Without Replacement

We know that under SRSWOR,

$$V(\bar{y}) = \frac{\sigma^2}{n} \frac{N-n}{N-1}$$

So, under SRSWOR:

$$d^2 = t_\alpha^2 \frac{p(1-p)}{n} \frac{N-n}{N-1}$$

$$n = \frac{NP(1-P)}{(N-1)D^2 + P(1-P)}$$

where,

$$D = d / t_\alpha$$

For continuous data,

$$n = \frac{N\sigma^2}{(N-1)D^2 + \sigma^2}$$

Alternative Specification (in two-stages):

This n , say n' , is estimated under simple random sampling with replacement (SRSWR). When sampling is without replacement, we adjust the n by

$$n = \frac{n'}{1 + \frac{n'}{N}}$$

or,

$$n = \frac{1}{\frac{1}{n'} + \frac{1}{N}}$$

(n is adjusted for finite population correction factor, $1 - n/N$).

Example: For exercise study, 93 samples are needed.

Say, the population size is 200.

Under SRSWOR:

$$n = \frac{n'}{1 + \frac{n'}{N}}$$
$$n = \frac{93}{1 + \frac{93}{200}} = \frac{93}{1 + 0.465} = \frac{93}{1.465} = 63.48$$
$$n \approx 64$$

Smaller sample size is needed when population size is small, but opposite is not true

Derivation

(alternative two-stage formula):

Remember the relationship between

$$\frac{S^2}{n'} \dots \text{vs} \dots \frac{N-n}{N} \frac{S^2}{n}$$

$$\Rightarrow \frac{1}{n'} = \left(1 - \frac{n}{N}\right) \frac{1}{n}$$

$$\Rightarrow \frac{1}{n'} = \frac{1}{N} - \frac{1}{n}$$

$$\Rightarrow \frac{1}{n} = \frac{1}{n'} + \frac{1}{N}$$

$$\Rightarrow \frac{1}{n} = \frac{1}{n'} + \frac{n'}{N n'}$$

$$\Rightarrow \frac{1}{n} = \frac{1 + \frac{n'}{N}}{n'}$$

$$\Rightarrow n = \frac{n'}{1 + n'/N}$$

Sample Size Based on Coefficient of Variation

- In the above, the sample size is derived from an *absolute* measure of variation, σ^2 .
- Coefficient of variation (cv) is a *relative* measure, in which units of measurement is canceled by dividing with mean.
- Coefficient of variation is useful for comparison of variables.

Coefficient of variation is defined as,

$$C_Y = \frac{S_y}{\bar{Y}}, \text{ and is estimated by } c_y = \frac{s_y}{\bar{y}}$$

Coefficient of variation (CV) of mean is

$$CV = \frac{SE}{\bar{y}} = \frac{s / \sqrt{n}}{\bar{y}} = \frac{s}{\bar{y}} \frac{1}{\sqrt{n}}$$

So,
$$n = \frac{1}{CV^2} \frac{s^2}{\bar{y}^2}$$

For proportion p ,

$$n = \frac{1}{CV^2} \frac{p(1-p)}{p^2} = \frac{1}{CV^2} \frac{(1-p)}{p}$$

Caution about using coefficient of variation (CV)

- If mean of a variable is close to zero, CV estimate is large and unstable.
- Next, consider CV for binomial variables. For binary variables, the choice of P and $Q=1-P$ does not affect $P(1-P)$ estimate, but CV differs. So, the choice of P affects sample size when CV method is used.

Cost considerations for sample size

How many samples you may afford to interview, given then budget constraints?

$C(n)$ = cost of taking n samples

c_o = fixed cost

c_1 = cost for each sample interview

then,

$$C(n) = c_o + c_1 \times n$$

Example:

$C(n)$ = \$10000 - your budget for survey implementation

c_o = \$3000 - costs for interviewer training, questionnaire prints,

etc

c_1 = \$8.00 - cost for each sample interview

$$10000 = 3000 + 8 \times n$$

So,

$$n = 875$$

Objective 2: Issues of Power Calculation

POWER

The power of a test is the probability of rejecting the null hypothesis if it is incorrect.

TRICKS to REMEMBER:

R, T: Reject the null hypothesis if it is true - Type I error (alpha error) { one stick in R, T}

A, F: Accept the null hypothesis if it is false - Type II error (beta error) {two sticks in A, F}

POWER: 1- type II error

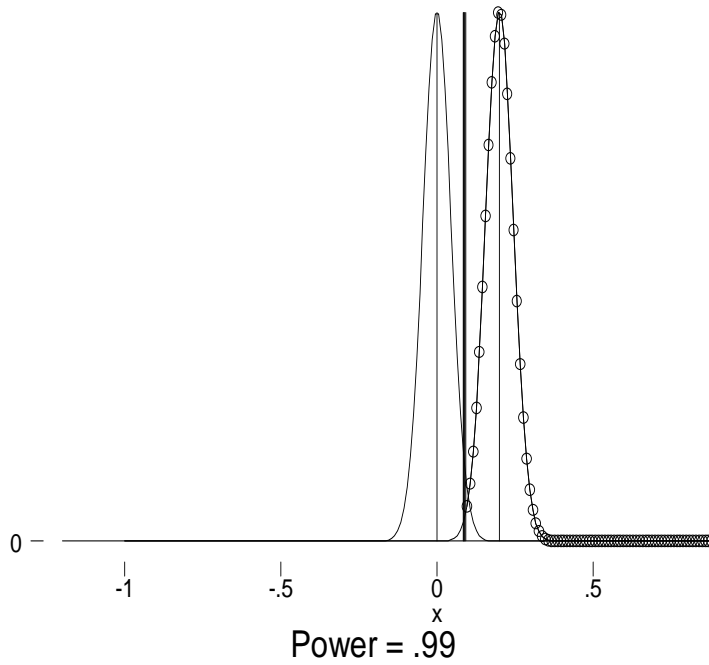
Power: Reject the null hypothesis if it is false.

Another way:

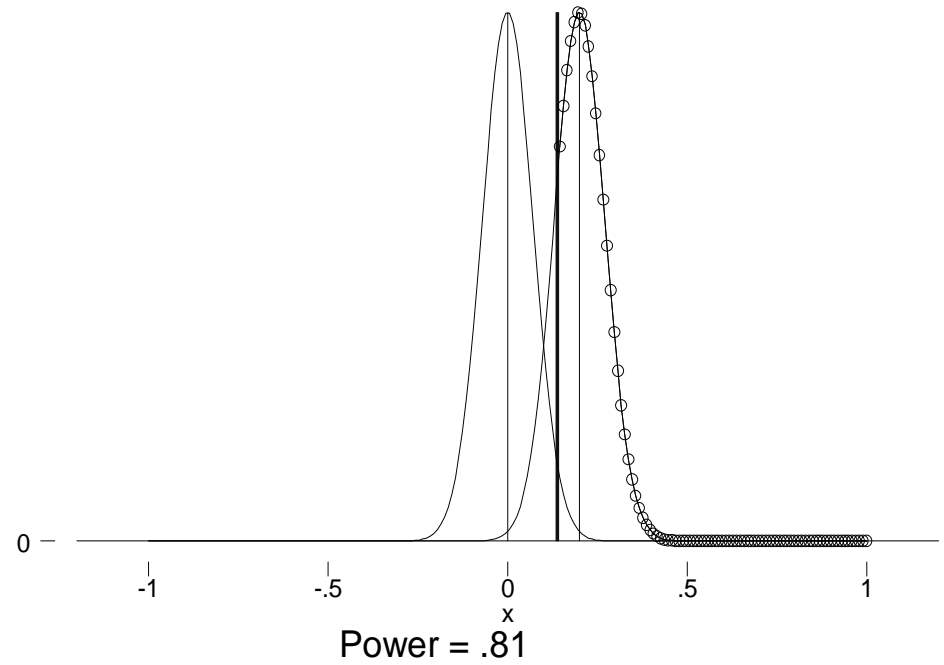
False Positive (YES instead of NO) ?

False Negative (NO instead of YES) ?

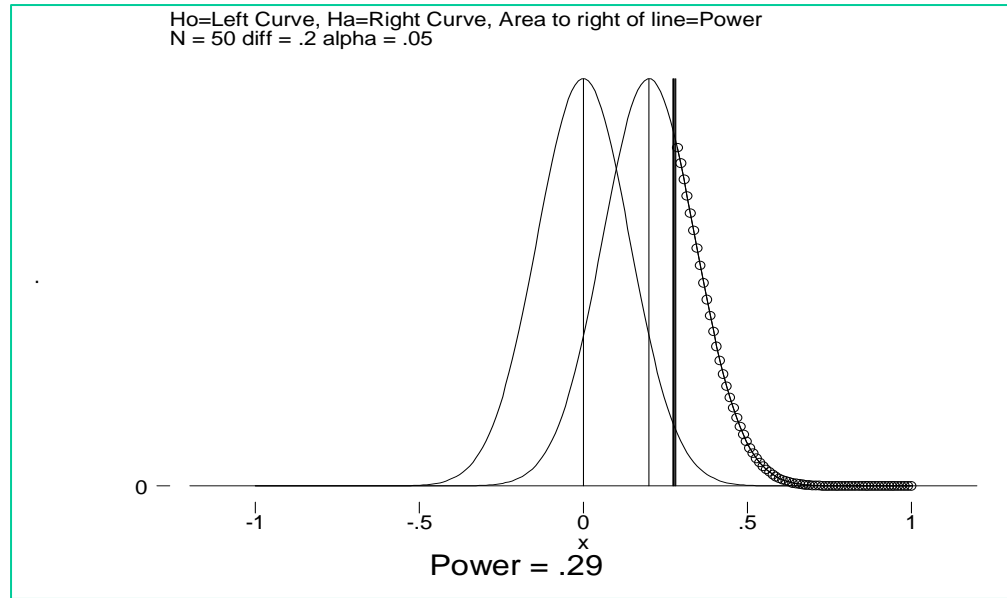
H_0 =Left Curve, H_a =Right Curve, Area to right of line=Power
N = 500 diff = .2 alpha = .05



H_0 =Left Curve, H_a =Right Curve, Area to right of line=Power
N = 200 diff = .2 alpha = .05



H_0 =Left Curve, H_a =Right Curve, Area to right of line=Power
N = 50 diff = .2 alpha = .05



We take power into consideration when we test hypotheses.

Example:

Consider following study questions:

1. What proportions of pregnant women received antenatal care?

There is no hypothesis.

b) Whether 80% of women received antenatal care?

There is a hypothesis:

To test that the estimated value is greater, less, or equal to a pre-specified value.

c) Do women in project (intervention) area more likely to utilize antenatal care, compared to control area?

There is a hypothesis:

To test that that P_1 is greater than P_2 .

In terms of hypothesis:

Null hypothesis: $H_0: P_1 = P_2$, i.e., $P_1 - P_2 = 0$

Alternative hypothesis: $H_a: P_1 > P_2$ (one-sided)

$H_a: P_1 \neq P_2$ (two-sided) i.e., $P_1 - P_2 \neq 0$

Issues:

One-sided vs. two-sided tests.

One-sided: sample size will be smaller.

Two-side: sample size will be larger.

Always prefer "two-sided" - almost a mandatory in clinical trials.

Why? Uncertainty in knowledge (*a priori*).

How to incorporate "power" in sample size calculations?

1. Proportions:

$$n = \frac{(t_{\alpha} + t_{\beta})^2 \bar{p}(1 - \bar{p})}{d^2}$$

where \bar{p} is $(p_1 + p_2) / 2$

Note: n for each group.

Alternative:

$$n_1 = n_2 = \left[\frac{Z_{\alpha} + Z_{\beta}}{2 \arcsin \sqrt{p_1} - 2 \arcsin \sqrt{p_2}} \right]^2$$

Why?

Arcsin provides normal approximation to proportion quantities.

For continuous variables:

$$n = \frac{(Z_{1-\alpha/2} + Z_{\beta})^2 s^2}{d^2}$$

Values of $Z_{1-\alpha/2}$ and Z_β corresponding to specified values of significance level and power

	Values	Two-sided	One-sided
Level	1%	2.576	2.326
	5%	1.960	1.645
	10%	1.645	1.282
Power	80%	0.84	
	90%	1.282	
	95%	1.645	
	99%	2.326	

How to incorporate "power" in sample size calculations?

a) Proportions:

$$n = \frac{(z_{(1-\alpha/2)} + z_{1-\beta})^2 \text{variance of difference}[\text{var}(p_1 - p_2)]}{(p_1 - p_2)^2}$$

How to estimate variance of difference?

$$\sigma_{(p_1 - p_2)}^2 = \sigma_d^2 = \sigma_{p_1}^2 + \sigma_{p_2}^2 - 2\sigma_{p_1} \sigma_{p_2}$$

Under the assumption of independence, $\text{cov}(p_1, p_2) = \sigma_{p_1} \sigma_{p_2} = 0$

If we also assume that $\text{var}(p_1) = \text{var}(p_2) = \text{var}(p)$, i.e., have common variance

$$\sigma_d^2 = \sigma_p^2 + \sigma_p^2 = 2\sigma_p^2$$

So,

$$n = \frac{(z_{(1-\alpha/2)} + z_{1-\beta})^2 \text{variance of difference}[v(p_1 - p_2)]}{(p_1 - p_2)^2}$$
$$= \frac{(z_{(1-\alpha/2)} + z_{1-\beta})^2 2 * \bar{p}\bar{q}}{(p_1 - p_2)^2} \text{ where } \bar{p} = (p_1 + p_2)/2$$

under the assumption of common variance

The sample size formula for testing two proportions under independence without the assumption of common variance is then:

$$n_1 = n_2 = \frac{(z_{1-\alpha/2} + z_{1-\beta})^2 [p_1(1-p_1) + p_2(1-p_2)]}{(p_1 - p_2)^2}$$

Note that Fleiss (1981) suggested more precise formula:

$$n' = \frac{\left\{ z_{1-\alpha/2} \sqrt{2\bar{p}(1-\bar{p})} + z_{1-\beta} \sqrt{p_1(1-p_1) + p_2(1-p_2)} \right\}^2}{(p_1 - p_2)^2}$$

$$\text{where, } p = (p_1 + p_2) / 2$$

When n_1 and n_2 is not equal and related by a ratio, say by r , the formula is:

$$n' = \frac{\left\{ z_{1-\alpha/2} \sqrt{(r+1)\bar{p}(1-\bar{p})} + z_{1-\beta} \sqrt{rp_1(1-p_1) + p_2(1-p_2)} \right\}^2}{r(p_1 - p_2)^2}$$

The final formula (using normal approximation with continuity correction [without the correction, the power is considered low than expected] with proportions) is:

$$n_1 = \frac{n'}{4} \left\{ 1 + \sqrt{1 + \frac{2(r+1)}{n'r |p_1 - p_2|}} \right\}^2$$

$$n_2 = rn_1$$

The STATA has implemented this formula in SAMPSI command.

Stata implementation

NO Hypothesis

```
. sampsi .5 .55, p(.5) onesample
```

Estimated required sample size:

n = 385

Study has a hypothesis, but comparing with a hypothesized value

```
. sampsi .5 .55, p(.8) onesample
```

Estimated sample size for one-sample comparison of proportion to hypothesized value

Estimated required sample size:

n = 783

Study has a hypothesis, and comparing between two groups

```
. sampsi .5 .55, p(.8)
```

Estimated sample size for two-sample comparison of proportions

n1 = 1605

n2 = 1605

```
. di 783/385  
2.0337662
```

```
. di  
(1.96+.84)^2/1.96^2  
2.0408163
```

Stata implementation

```
. sampsi .5 .55, p(.8) nocontinuity
```

Estimated sample size for two-sample comparison of proportions

Test Ho: $p_1 = p_2$, where p_1 is the proportion in population 1
and p_2 is the proportion in population 2

Assumptions:

alpha = 0.0500 (two-sided)

power = 0.8000

$p_1 = 0.5000$

$p_2 = 0.5500$

$n_2/n_1 = 1.00$

Estimated required sample sizes:

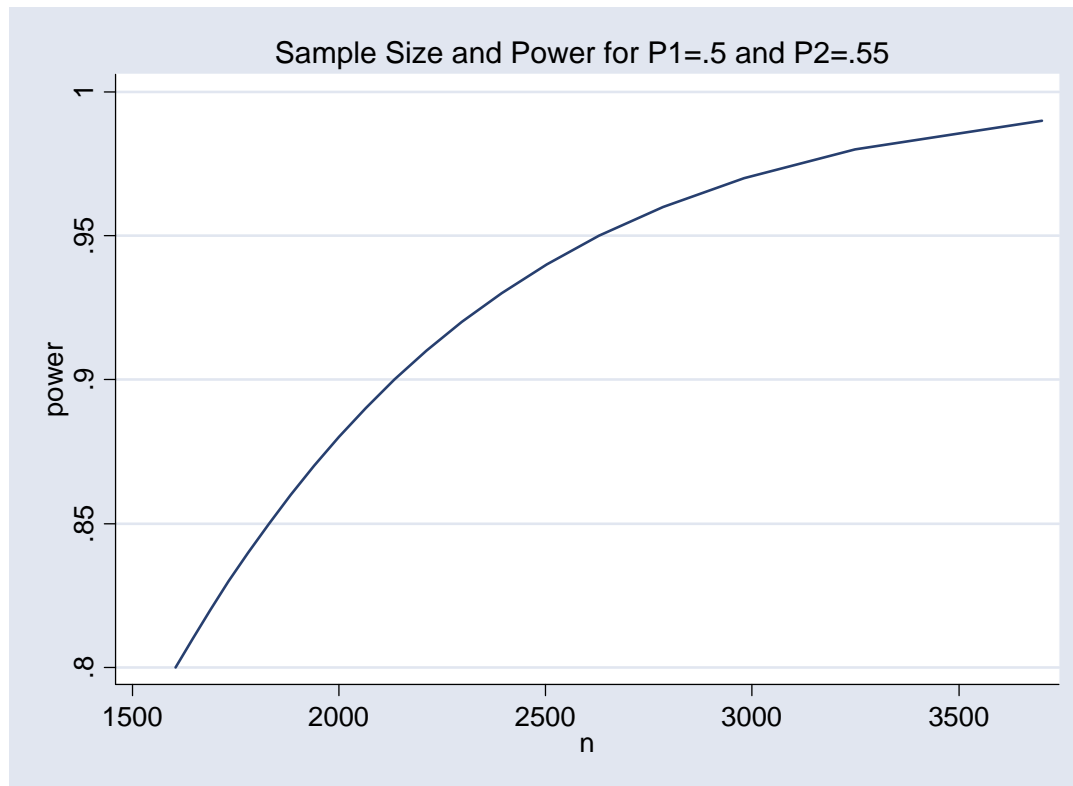
n1 = 1565

n2 = 1565

```
. di 783*2  
1566
```

In each group,
sample size is
doubled

Power graph in Stata



*Calculate and plot sample size by power from .8 to .99

```
*****
```

```
args p1 p2 type
```

```
clear
```

```
set obs 20
```

```
gen n=.
```

```
gen power=.
```

```
local i 0
```

```
while `i' <_N {
```

```
    local i = `i' +1
```

```
    local j=.79 + `i'/100
```

```
    quietly sampsi `p1' `p2', p(`j') `type'
```

```
    replace n=r(N_1) in `i'
```

```
    replace power=r(power) in `i'
```

```
}
```

```
noisily list power n
```

```
graph twoway line power n, t1("Sample Size and Power for P1=`p1' and P2=`p2' `type'")
```

```
*****
```

Save the above commands as “do” file (e.g., sample_graph.do).

Execute the above file by:

```
run sample_graph
```

Sample size determination when expressed in “relative risk”

In epidemiological studies, often the hypothesis is expressed in relative risk or odds ratio, e.g, $H_0:R=1$.

A sample size formula given in Donner (1983) for Relative Risk (p. 202) is:

$$n = \left\{ Z_{\alpha} \sqrt{2\bar{P}_R(1 - \bar{P}_R)} + Z_{\beta} \sqrt{P_c \{1 + R - P_c(1 + R^2)\}} \right\}^2 / [P_c(1 - R)]^2$$

Where $\bar{P}_R = [P_c(1 + R)]/2$ and $R = P_E / P_c$

Nothing but the Fleiss' formula:

$$n' = \frac{\left\{ z_{1-\varepsilon} \sqrt{2\bar{p}(1-\bar{p})} + z_{1-\beta} \sqrt{p_E(1-p_E) + p_C(1-p_C)} \right\}^2}{(p_C - p_E)^2}$$

where, $p = (p_E + p_C) / 2$

Note, $R = \frac{P_E}{P_C} = P_E = RP_C$

Solution: Replace all P_E with RP_C and apply Fleiss' formula

How Donner's formula was derived:

$$\begin{aligned} P &= (P_E + P_C) / 2 = (RP_C + P_C) / 2 = [P_C(R + 1)] / 2 = [P_C(1 + R)] / 2 \\ P_E(1 - P_E) + P_C(1 - P_C) &= RP_C(1 - RP_C) + P_C(1 - P_C) \\ &= RP_C - R^2P_C^2 + P_C - P_C^2 \\ &= P_C(R - R^2P_C + 1 - P_C) \\ &= P_C(1 + R - P_C(1 + R^2)) \end{aligned}$$

and, $(P_C - P_E)^2 = (P_C - RP_C)^2 = [P_C(1 - R)]^2$

Sample size for odds-ratio (OR) estimates:

$$OR = \frac{\frac{P_2}{1 - P_2}}{\frac{P_1}{1 - P_1}} = \frac{P_2 Q_1}{P_1 Q_2}$$

$$P_2 Q_1 = OR * P_1 Q_2 = OR * P_1 (1 - P_2) = OR * P_1 - OR * P_1 * P_2$$

$$P_2 (Q_1 + OR * P_1) = OR * P_1$$

$$P_2 = \frac{OR * P_1}{OR * P_1 + Q_1}$$

Convenient to do in two stages:

1. Estimate P_2 from odds-ratio (OR)
2. Apply “proportion method” (of Fleiss)

An example

Suppose we want to detect an OR of 2 using an ratio of 1:1 cases to controls in a population with expected exposure proportion in non-cases of 0.25 while requiring a $\alpha = 0.05$ and power = 0.8.

How to estimate SS?

EpiTable calculates $m_1 = m_2 = 165$. (Total sample size = 330).

So, $P_1 = .25$,

$$P_2 = (2 * .25) / (2 * .25 + .75) = 0.4$$

In Stata:

```
. sampsi .25 .40, p(.8)
```

Estimated required sample sizes:

$$n_1 = 165$$

$$n_2 = 165$$

SAMPLE SIZE determination for Logistic Regression Models

Consider a logistic regression,

$$\log\left(\frac{p}{1-p}\right) = \text{logit}(p) = \alpha + \beta x$$

We want to estimate sample size needed to achieve certain power for testing null hypothesis

$H_0: \beta=0$. Recall that null hypothesis testing depends on the **variance of β** . In logistic regression, the **effect size** is expressed as “log odds ratio” (η).

Hsieh(1989) suggested the following formula for one-sided test:

$$n = \left[z_{\alpha} + z_{\beta} \exp(-\eta^2 / 4) \right]^2 (1 + 2\hat{p}\delta) / (\hat{p}\eta^2)$$

where,

$$\delta = [1 + (1 + \eta^2) \exp(5\eta^2 / 4)] / [1 + \exp(-\eta^2 / 4)]$$

Say, you want to examine the **effect size of log odds ratio** of $1.5 = \log(1.5) = .40546511 = \sim 0.41$

See, the implementation of formula in STATA:

```
. clear
. set obs 1
obs was 0, now 1

. *Enter "odds-ratio"
. gen p=0.10
. gen or=1.5

. gen beta=log(or)

. di beta
.4054651
. gen delta= (1+(1+beta^2)*exp(5*beta^2/4))/(1+exp(-beta^2/4))
.
. di delta
1.2399909
.
. di " n = " (1.645+1.282*exp(-
beta^2/4))^2*(1+2*p*delta)/(p*beta^2)
n = 627.61987
```

So, **n = 628 = ~ 630**

Sample Size for Multiple logistic Regression

Multiple logistic regression requires larger n to detect effects. Let R denote the multiple correlation between the independent variable of interest, X, and the other covariates.

Then, sample size:

$n^* = n/(1-R^2)$
Say, if $R=0.25$, then $n^* = 630/(1-0.25^2) = 672$

Stata's add-on programs for sample size estimation

- STPOWER: Survival studies
- Sampsi_reg: Linear regression
- Sampclus: Cluster sampling
- ART: randomized trials with survival time or binary outcome
- XSAMPSI: Cross-over trials
- **Samplesize**: Graphical results
- MVSAMPSI: multivariate regression

- **STUDYSI**: Comparative study with binary or time-to-event outcome
- **SSKAPP**: Kappa statistics measure of inter-rater agreement
- **CACLSI**: log-rank/binomial test

Additional topics to be covered

- Sample allocation – stratified sampling
- Sample size corrected for design-effect(DEFF)
- Optimal sample size per cluster
- Sample size for clusters
- Sample size and power for pre-post surveys in program evaluation