Medical Statistician Presentation

NTNU Interview Case

Artem Shiryaev

2023-11-26

Research Problem

Vi skal genomföre en klinisk studie på nydiagnostiserte pasienter med myelomatose hvor vi ska sammenlign standardbehandling med en ny kombinasjon av experimentella medikamenter.

Fra tidigare studier kan forvente:

- Standard: 50% minimal residual disease (MRD)

Forskargruppe tror att vi kan oppnå en klinisk signifikant bedring om:

- Experimentell: 70% minimal residual disease (MRD)

Med andre ord, jo mindre kreft, desto bedre.

Statistical Analys

Head of the Research team thinks: Data costs money,

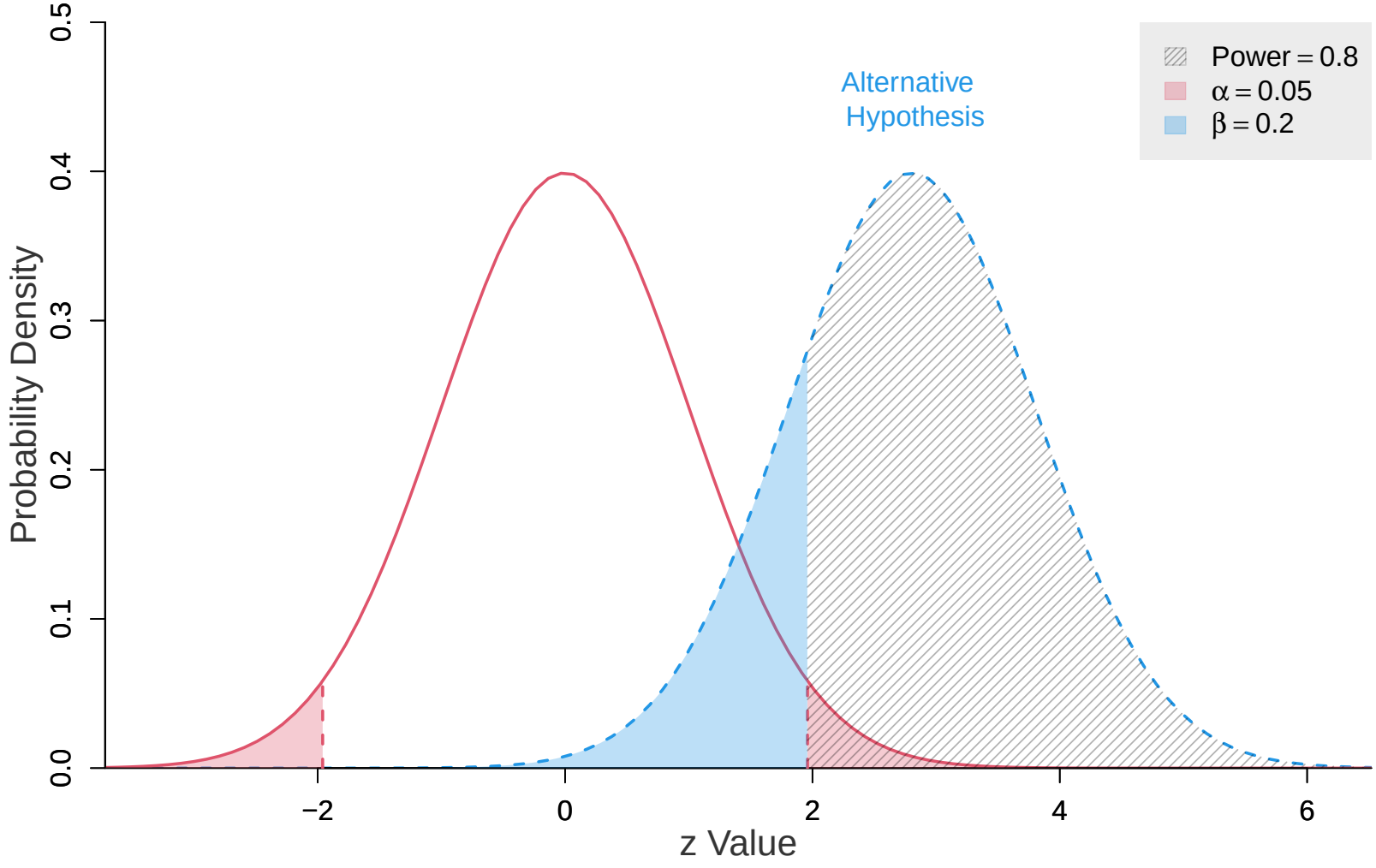
Hvor mange patienter trenger studien vår, med 80% power og signifikansnivå på 5 %?

Resultat

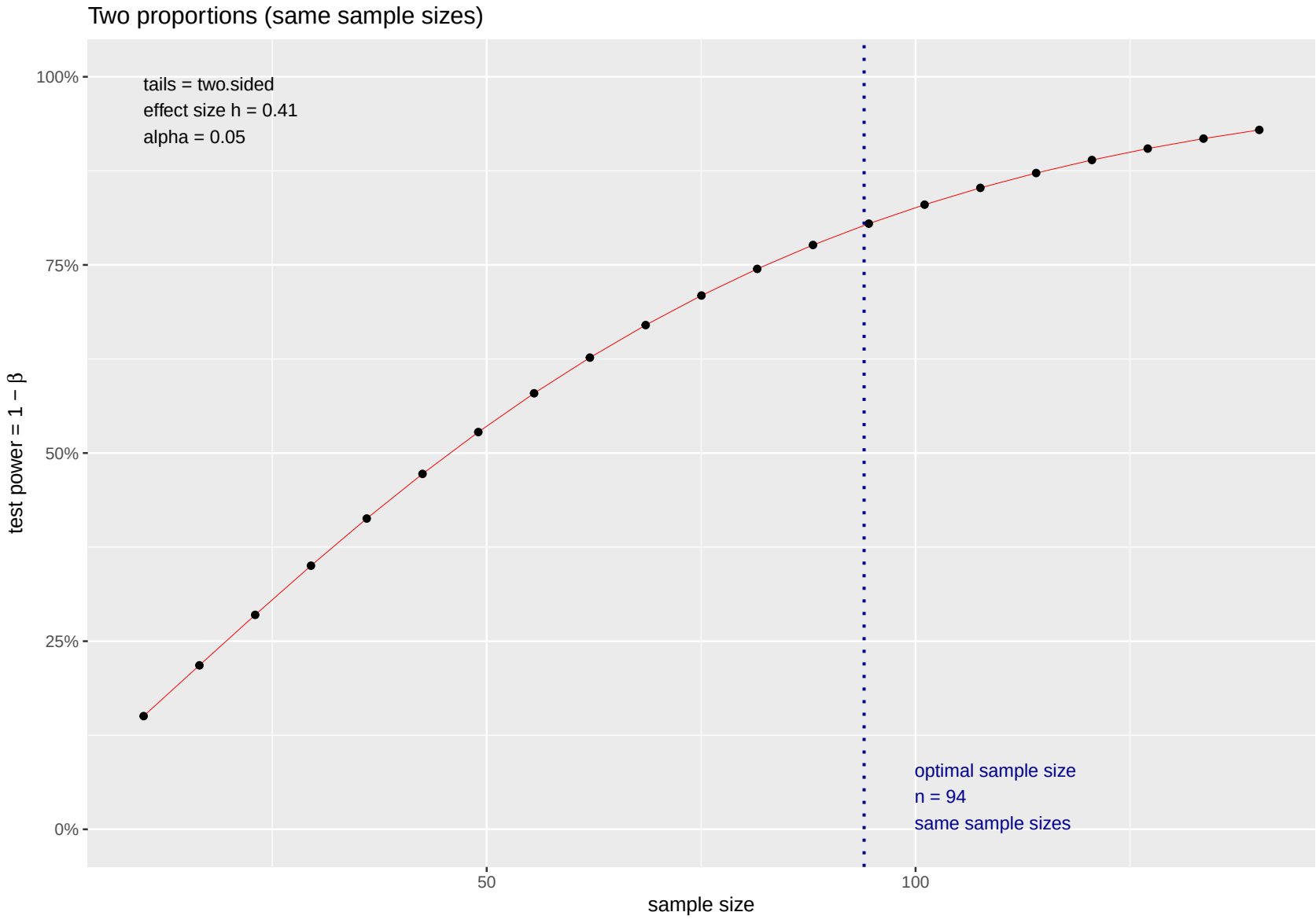
Beste fall: total 182 patienter, 91 vardera gruppe (standard/experiment) Verste fall: 231 Details:

Test	Same	Different	Assumptions
Two Sample Z	$91 + 91$	-	Normality, iid
Two Sample T	$93 + 93$	$180 + 63$	Approx. Normal, iid
Mann-Whitney U	$99 + 99$	-	Non-parametric, iid, effect size ≈ 0.41
Chi^2 ($df = 90$)	231	-	Z scores for df

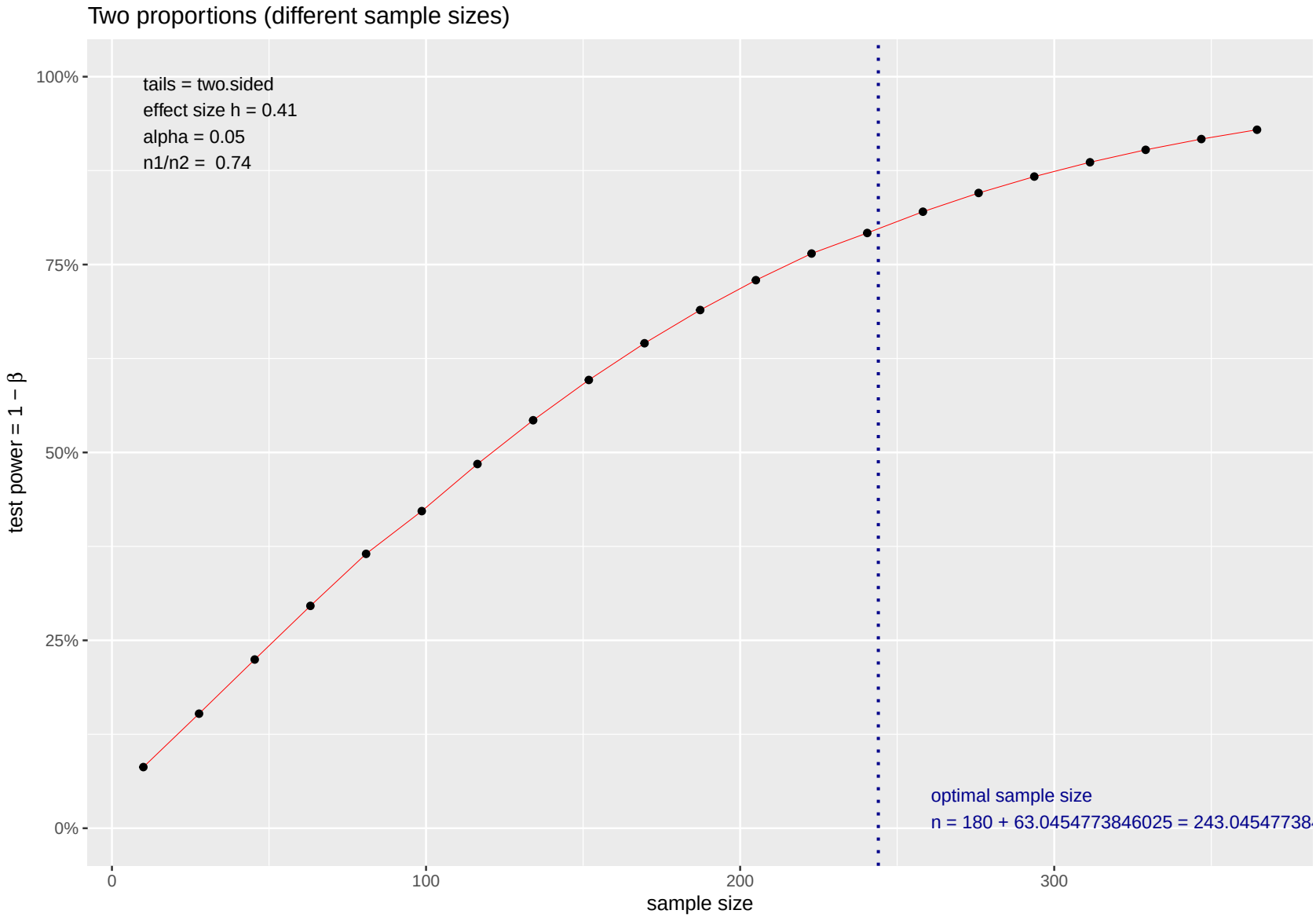
Graphical Results Z Test



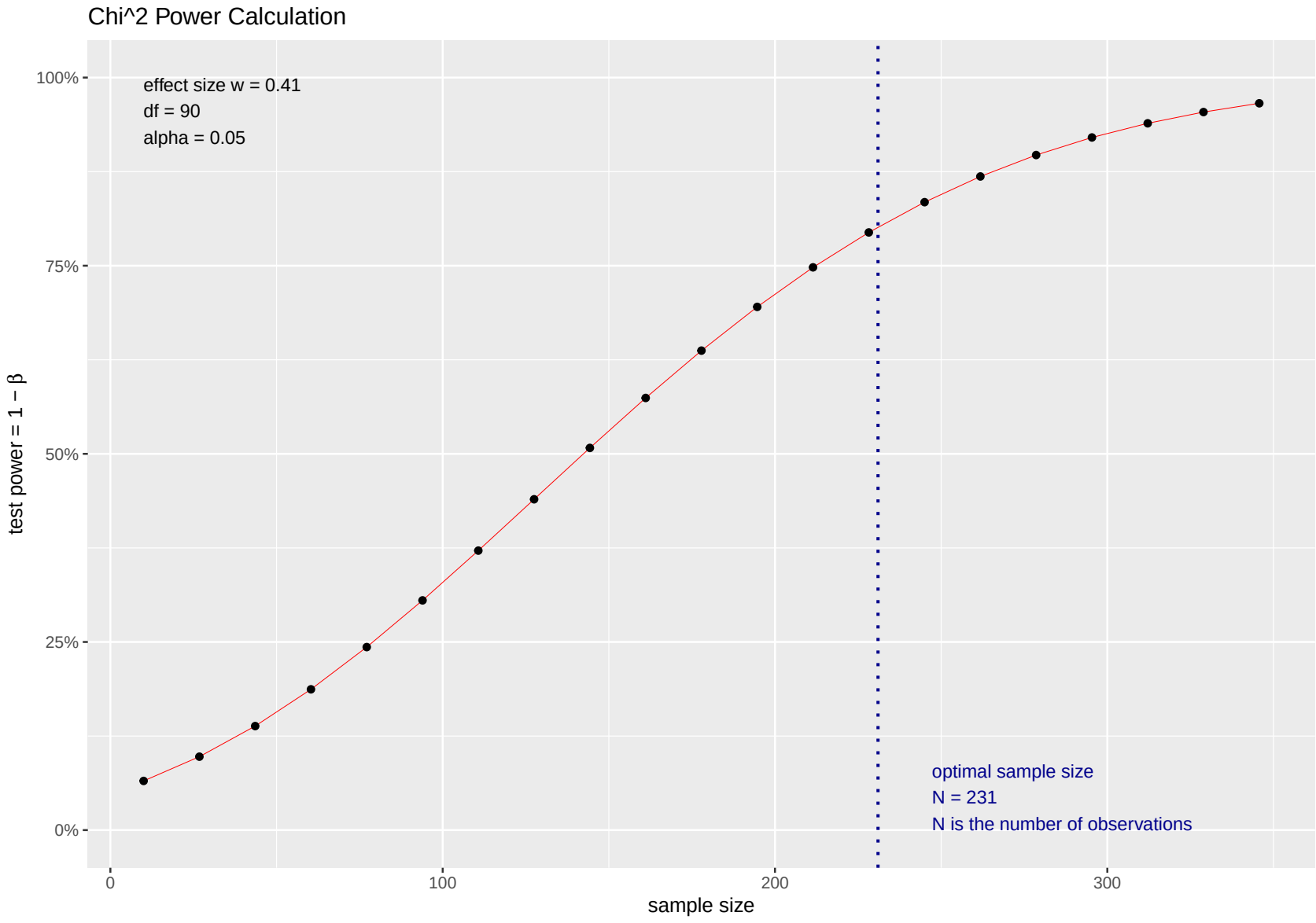
Graphical Results Student t-test



Graphical Results Student t-test



Graphical Results Chi^2



Code

```
library(pwr)
library(pwrss)
library(ggplot2)
library(scales)



Prop_DrugA <- 0.5
Prop_DrugB <- 0.7

Alpha <- 0.05 # Significance Level
Beta <- 0.8   # Power Level

# Difference between Two Proportions (z Test)
z_prop <- pwrss.z.2props(p1 = Prop_DrugA,
                         p2 = Prop_DrugB,
                         alternative = "not equal",
                         power = Beta,
                         alpha = Alpha
```

Analytical Calculation: Z-tests

$$H_0 : p_1 = p_2$$

$$H_1 : p_1 \neq p_2$$

To determine the sample size needed for a study comparing two proportions use two-sample proportion test. The formula is:

$$Z = \frac{(p_1 - p_2)}{\sqrt{\frac{p_1(1-p_1)}{n_1} + \frac{p_2(1-p_2)}{n_2}}}$$

Simplified to, given $n_1 = n_2 = n$:

$$n = \frac{(p_1(1-p_1) + p_2(1-p_2))(Z_\alpha + Z_\beta)^2}{(p_1 - p_2)^2}$$

Analytical Calculation: Z-tests

p_1 is the reduction in MRD for Drug A (50%, or 0.5). p_2 is the targeted reduction in MRD for Drug B (70%, or 0.7). Z_α is the Z-score for a 5% significance level (approximately 1.96). Z_β is the Z-score for an 80% power (approximately 0.84).

Analytical Calculation: Z-tests

Substitute these values into the formula to calculate n:

$$n = \frac{(0.5 \times 0.5 + 0.7 \times 0.3)(1.96 + 0.84)^2}{(0.5 - 0.7)^2}$$

Now, calculate the result.

$$n = \frac{(0.25 + 0.21)(2.8)^2}{0.04}$$

$$n = \frac{0.46 \times 7.84}{0.04}$$

$$n = \frac{3.6064}{0.04}$$

$$n = 90.16$$

$$n \approx 91$$

Analytical Calculation: Student t-test

test