Superiority Trials

compare two treatment groups on a continuous outcome

formally test	H_0: $\mu_A = \mu_P$	H_A: $\mu_A \neq \mu_P$
design the study to show	H_0: $\mu_A = \mu_P$	H_A: $\mu_A < \mu_P$

by convention, test H_0 against a two-sided H_A

Sample Size Calculations of Continuous Outcomes

$$n \geq \frac{2\left(Z_{1-\frac{\alpha}{2}} + Z_{1-\beta}\right)^2 \sigma^2}{\Delta^2}$$

σ	outcome standard error, same for each group
$\Delta = \mu_A - \mu_P$	expected mean difference
$Z_{1-\frac{\alpha}{2}}$	critical value corresponding to significance level α, usually 1.96
$Z_{1-\beta}$	standard normal value not exceeded with probability β, usually 0.84

At least n subjects per group, or kn subjects total are needed to have an 80% chance to detect a significant mean difference of Δ or more. There's still a 20% chance there is an unsuccessful trial even if all the assumptions are correct.

if σ^2 is underestimated, the study is underpowered, but might still find significant results
if Δ^2 isn't as large as assumed, study might be underpowered
if n is overestimated, the trial might be unfeasible
if n is underestimated, the trial won't be able to demonstrate difference between groups
better to overestimate and terminate the trial earlier than to underestimate
sample size accounting for f loss to follow-up = $\frac{n}{1-f}$

Sample Size Calculations of Binary Outcomes

$$n \geq \frac{\left(Z_{1-\frac{\alpha}{2}} + Z_{1-\beta}\right)^2 2\bar{p}\bar{q}}{(p_A - p_P)^2}$$

$$\bar{p} = \frac{p_A - p_P}{2}$$

$$\bar{q} = 1 - \bar{p}$$

$Z_{1-\frac{\alpha}{2}}$	critical value corresponding to significance level α, usually 1.96
$Z_{1-\beta}$	standard normal value not exceeded with probability β, usually 0.84

At least n subjects per group, or kn subjects total are needed to have an 80% chance to detect a significant mean difference of $p_A - p_P$ or more. There's still a 20% chance there is an unsuccessful trial even if all the assumptions are correct.

sample size accounting for f loss to follow-up = $\frac{n}{1-f}$