Types of Outcomes

continuous, binary, time to event, categorical, counts

not recommended to convert continuous to binary outcomes because arbitrary thresholds are set and information is lost

Continuous Outcomes

<u>Parametric Tests</u>

H_0: $\mu_A = \mu_B$

H_A: $\mu_A \neq \mu_B$

two-sided α=0.05 to allow if new drug is statistically more harmful than the control

two-sided α=0.05 requires larger sample size than one-sided α=0.05 level of significance

two-sample t-test compares mean of two treatment group with equal variances

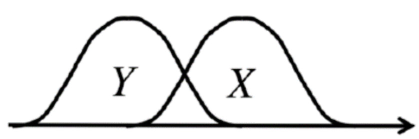
Welch's t-test compares means of two treatment group with different variances

<u>Non-Parametric Tests</u>

H_0: $median_A = median_B$

H_A: $median_A \neq median_B$

Wilcoxon rank-sum test compares median of two identical but shifted distributions



if the data is actually normally distributed, the Wilcoxon rank-sum test is 95% as effective as a t-test

Logarithmic Transformation

assumes that data follows a natural log distribution

can only be used with positive values

Bootstrap Confidence Intervals

obtain 95% confidence interval around the estimate for the original data

multiple resamples with replacement from a single set of observations

calculate effect size of interest on each resample and determine 95% confidence interval

resampling distribution approaches a normal distribution due to Central Limit Theorem

Binary Outcomes

determine if two or more treatments differ significantly with respect to the risk of the outcome
yes/no outcome, e.g. incidence of disease, cancer recurrence, hospitalization
H_0: $\pi_A = \pi_P$. The population risk for disease is the same for treatment and placebo groups.
H_A: $\pi_A \neq \pi_P$. The population risk for disease is not the same for treatment and placebo groups.

H_0: $p_A = p_P$. The risk for disease is the same for treatment and placebo groups.
H_A: $p_A \neq p_B$. The risk for disease is not the same for treatment and placebo groups.

	No Disease	Disease	
Placebo	a	b	m_0
Treatment	c	d	m_1
	n_0	n_1	n

Crude Measures of Association

$p_P = P(dis = 1, trt = P) = \frac{b}{m_0}$ = risk of disease in placebo group
$p_A = P(dis = 1, trt = A) = \frac{d}{m_1}$ = risk of disease in treatment group

Risk Difference

$$RD = P(dis = 1, trt = A) - P(dis = 1, trt = P) = \frac{d}{m_1} - \frac{b}{m_0}$$

There are $RD\%$ fewer cases of disease in the treatment group than in the placebo group.

H_0: $RD = 0$
The risk for the disease is the same for both treatment and placebo group.
H_A: $RD \neq 0$
There is a lower/higher risk for the disease in the treatment group than the placebo group.

Number Needed to Treat

$$NNT = \frac{1}{RD}$$

average number of individuals needed to treat to prevent 1 additional outcome event
NNT are needed to be treated in order to prevent 1 disease outcome event.

Risk Ratio/Relative Risk

$$RR = \frac{P(dis = 1, trt = A)}{P(dis = 1, trt = P)} = \frac{\frac{d}{m_1}}{\frac{b}{m_0}}$$

The risk of disease in the treatment group is $RR\%$ of the risk in the placebo group.

H_0: $RR = 1$

The risk for the disease is the same for both treatment and placebo group.

H_A: $RR \neq 1$

There is a lower/higher risk for the disease in the treatment group than the placebo group.

<u>Odds Ratio</u>

$$\frac{\frac{P(dis=1, trt=A)}{P(dis=0, trt=A)}}{\frac{P(dis=1, trt=P)}{P(dis=0, trt=P)}} = \frac{\frac{a}{b}}{\frac{c}{d}} = \frac{a}{b} \times \frac{d}{c} = \frac{ad}{bc}$$

The odds of the disease in the treatment group is OR% of the odds in the placebo group.
can only interpret OR as if it were RR when outcome is uncommon, <2%

H_0: $OR = 1$

The odds of the disease is the same for both treatment and placebo group.

H_A: $OR \neq 1$

There is a lower/higher odds of the disease in the treatment group than the placebo group.

Logistic Regression

$$logit(p) = log\left(\frac{p}{1-p}\right) = \beta_0 + \beta_1 X$$

$$p = \frac{e^{\beta_0 + \beta_1 X}}{1 + e^{\beta_0 + \beta_1 X}}$$

p = probability of the disease outcome
X = treatment indicator dummy variable

$X = 0$ for control group

$X = 1$ for treatment group

H_0: $\beta_1 = 0$

H_A: $\beta_1 \neq 0$

<u>Probabilities</u>

$$\hat{p}_P = \frac{e^{\beta_0 + \beta_1(0)}}{1 + e^{\beta_0 + \beta_1(0)}} = \frac{e^{\beta_0}}{1 + e^{\beta_0}}$$

$$\hat{p}_A = \frac{e^{\beta_0 + \beta_1(1)}}{1 + e^{\beta_0 + \beta_1(1)}} = \frac{e^{\beta_0 + \beta_1}}{1 + e^{\beta_0 + \beta_1}}$$

<u>Odds</u>

$$log\ odds_P = log\left(\frac{p}{1-p}\right) = \beta_0 + \beta_1(0) = \beta_0$$

$$odds_P = \left(\frac{p}{1-p}\right) = e^{\beta_0 + \beta_1(0)} = e^{\beta_0}$$

$$log\ odds_A = log\left(\frac{p}{1-p}\right) = \beta_0 + \beta_1(1) = \beta_0 + \beta_1$$

$$odds_A = \left(\frac{p}{1-p}\right) = e^{\beta_0+\beta_1(1)} = e^{\beta_0+\beta_1}$$

<u>Odds Ratio</u>

$$OR = \frac{odds_A}{odds_P} = \frac{e^{\beta_0+\beta_1}}{e^{\beta_0}} = e^{\beta_1}$$