Study Design and Analysis in Epidemiology II: RCT's

Lab 4 Summary

Goals

- Simulate data
  - Using a function
- Understanding Type I Error
- Power
- Inference
- Exploring some clinical trial data
  - Generalised Linear Models
- Summary

Generating a simulated data set

Study ID	Treatment	Outcome
1	1	1.0385574
2	0	0.4923728
3	0	1.2503000
4	1	1.3051281
⋮	⋮	⋮
17	0	1.0349434
18	0	1.2704913
19	1	1.143064

Function

- Number of subjects : n.Subjects
- Mean : control.mean
- Outcome : treatment.effect
- Standard deviation : sd

Understanding Type I Error

False positive

Two sample t-test - When $p < 0.05$, we reject the null hypothesis at a significance level of 0.05.

- Increasing the number of subjects

  The type 1 error **does not** change

Power

False negative

The probability of rejecting the null hypothesis when you should reject it

- Increasing the number of subjects

  Power **does** change

- Changing the difference between the treatment group and the control group

  Lower the treatment effect - the power goes down

Exploring some clinical trial data

Mycotic Ulcer Therapeutic Exploratory Trial

- patient ID
- drug assignment: 0 is natamycin, 1 is voriconazole
- scraping: 0 is no, 1 is yes
- age
- sex
- Perforation: 1 if a perforation happened
- scar size at baseline
- scar size at 3 weeks

Estimate the treatment effect on scar size at 3 weeks.

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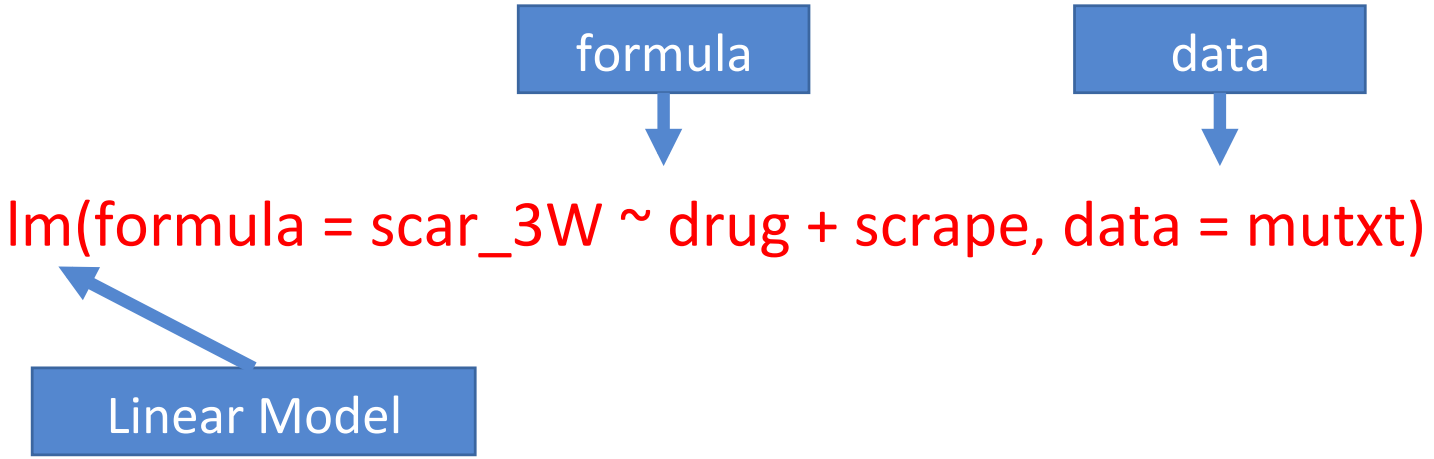
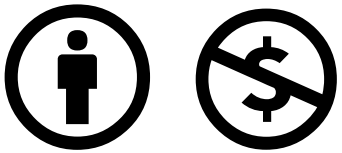


	Estimate	Std. Error	t value	Pr(>|t|)
(Intercept)	4.14922	0.30834	13.457	<2e-16 ***
drug	-0.07211	0.35809	-0.201	0.841
scrape	0.15285	0.35804	0.427	0.670

Summary

- RCTs greatly reduce the chance that confounding is present via randomization of treatment assignment
- RCTs provide us with causal evidence.
- Masking can greatly reduce bias in RCTs. The lack of masking usually leads to inflated estimates of treatment effects
- Oversight reduces exploratory data analysis, preserves the integrity of the research, and protects participants



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