Course 3 · Week 1 — Designing studies

Cheatsheet — biostats_courses

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Observational designs

Design	Strengths	Weaknesses
Cohort	temporality, incidence	expensive, loss to follow-up
Case-control	rare outcomes, cheap	recall + selection bias
Cross-sectional	prevalence, screening	no temporality
Case-crossover	within-person comparison	for transient exposures only

STROBE checklist: https://www.strobe-statement.org/

Trial designs

Type	When
Parallel-group	most common; independent arms
Crossover	stable chronic condition, carry-over washable
Cluster	intervention at group level (schools, clinics)
Factorial	two or more interventions, test interactions
Adaptive	pre-specified modifications based on interim data
Non-inferiority	new treatment not worse than control by margin Δ
Equivalence	two-sided non-inferiority

CONSORT for RCTs: https://www.consort-statement.org/

Bench / translational

- **Blocking** reduces nuisance variation (plate, day, operator).
- **Factorial** tests interactions efficiently.
- **Split-plot** handles two levels of randomisation.
- **Pseudoreplication**: technical replicates ≠ biological replicates.

Power — closed form

```
library(pwr)
pwr.t.test(d = 0.5, power = 0.80, sig.level = 0.05,
           type = "two.sample")
pwr.2p.test(h = ES.h(0.3, 0.2), power = 0.80)
pwr.r.test(r = 0.3, power = 0.80)
pwr.anova.test(k = 4, f = 0.25, power = 0.80)
```

Effect-size conventions (Cohen): small d = 0.2, medium 0.5, large 0.8.

Power — simulation

```
library(simr)
# Build a pilot model, increase N, or tweak fixef, then:
ps <- powerSim(model, nsim = 500, test = fixed("arm"))
ps
```

Simulation wins for any design the textbook skips: mixed models, adaptive rules, non-standard outcomes.

Decision rule for Week 1

- Randomise if you can. If not, draw a DAG and name the biases.
- Power calculation before the protocol freeze, not after data collection.
- Cluster randomisation → design effect $1 + (\bar{m} - 1)\rho$; inflate N.
- Bench experiments → treat batch, plate, and operator as random effects.

Common pitfalls

- Planning a cluster RCT without inflating N for design effect.
- Borrowing a pilot effect size without acknowledging noise.
- Running a non-inferiority trial as if it were superiority.
- Treating technical replicates as if they were biological.

Further reading

- Matthews, *Introduction to Randomized Controlled Clinical Trials*.
- Chow & Liu, *Design and Analysis of Clinical Trials*.