Choosing a study design to answer a specific research question

Clinical Research Design IT Statistical Services

Importance of study design
- Will determine how you collect, analyse and interpret your data
- Helps you decide what resources you need
- Impact on the reliability of your study results

Types of study design

Descriptive:
- Provides an overview of what is happening within a particular population or group
- Includes: cross-sectional and qualitative

Types of study design

Analytical:
- Quantify the relationship between two factors.
  1. Experimental designs:
     - Randomised controlled trials
     - Non-randomised trials
  2. Observational designs:
     - Cohort
     - Case-control
     - Cross-sectional
How to decide on a research design

• What is your research question?
• PICO
  • Patient or Problem or Population
  • Intervention
  • Comparison
  • Outcome

Reference:
BMJ 2010;340:c869 doi: 10.1136/bmj.c869
Intervention delivered
Observational
NO
Comparison
Descriptive
Yes
Analytic
Cross-sectional
Case-control
Cohort

How to decide on a research design

- What is your research question?
- What design is the most rigorous?

Levels of evidence

- Meta-analysis
- Systematic review
- Randomised controlled trials
- Cohort studies
- Case control studies
- Cross-sectional studies
- Case series/case reports

Reference:
BMJ 2010;340:c869 doi: 10.1136/bmj.c869
How to decide on a research design

• What is your research question?
• What design will give you the greatest level of evidence?
• What is ethically appropriate?

Pilot studies

• Small scale preliminary study of your larger trial
• Helps to establish:
  • Feasibility
  • Procedures and materials
  • Cost
  • Barriers and enablers
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Pilot studies ARE NOT
• An evaluation of effectiveness
• An assessment of the main hypotheses
• An indication of effect size

How will you reduce bias?
• Strategies to reduce error within your data.
• How to avoid confounding.
• Reduce threats to the validity of your study.

Gold standard RCT
• Control group
  • Assess direct effects of intervention
• Random allocation of participants
  • Ensures comparability of groups
  • Protects against selection bias

Well performed RCTs
• Well-defined inclusion and exclusion criteria
  • Homogenous to ensure comparability
  • Representative of the population
• Concealment of treatment allocation
  • Allocation should occur after baseline
• Blinding
  • Double-blind
  • Single-blind
• Intention to treat (ITT analysis)
**Limits of RCTs**

- Not always possible
- Can be expensive
- Reduced external validity (limitation believed by some)
- Ethically inappropriate

**When you can’t randomise individuals**

- Cluster randomised Controlled trials (CRCTs)
  - Randomise ‘clusters’ of individuals
- Advantages
  - Reduces contamination
- Design considerations
  - Unit of analyses
  - Number of clusters
  - Clustering effects
  - Sample size and analysis is more complex

**When you can’t randomise individuals**

- Stepped wedge design
  - ‘Clusters’ begin in the control and are randomly allocated to cross-over to the intervention at pre-determined sequences
- Advantages
  - Pragmatic
  - Provides a rigorous design where otherwise not possible
  - Efficient
- Design considerations
  - Clustering effect
  - Temporal effects
  - Number of clusters
  - Number and length of steps
  - Collect data at each time-point
  - Sample size and analysis more complex

**What to put in your research grant**

- Justify your design
- Justify why alternate designs were not chosen
- Identify the strengths but also the weaknesses of your design
- Offer solutions/strategies to address the weaknesses
- Demonstrate that the design is feasible
- Offer alternatives if things do not go as planned

**Useful guidelines/documents**

- CONSORT Statements and checklists
- STROBE
- Cochrane – risk of bias
- RE-AIM Framework
**Things to remember**

- Select a design that allows you to answer your research question
- Select a design that provides the highest level of evidence possible – but is also feasible
- Conduct a pilot
- Pay attention to the finer details

**Useful references**