Evaluating Intervention Evidence

Evidence-Based Practice in Speech-Language Therapy
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Session 3

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Outline

1. Hierarchy of evidence
2. Randomised controlled trials (RCTs)
3. Reporting standards
4. Critically appraising evidence
5. Group discussion

Hierarchy of Evidence

Hierarchy of evidence

- Searching for evidence can turn up anything from well-designed research studies to expert opinions to personal opinions.
- Various schemes have been designed to rank the scientific value of different kinds of evidence.
- Consider the strength of evidence.

Simple hierarchy of intervention evidence[1]

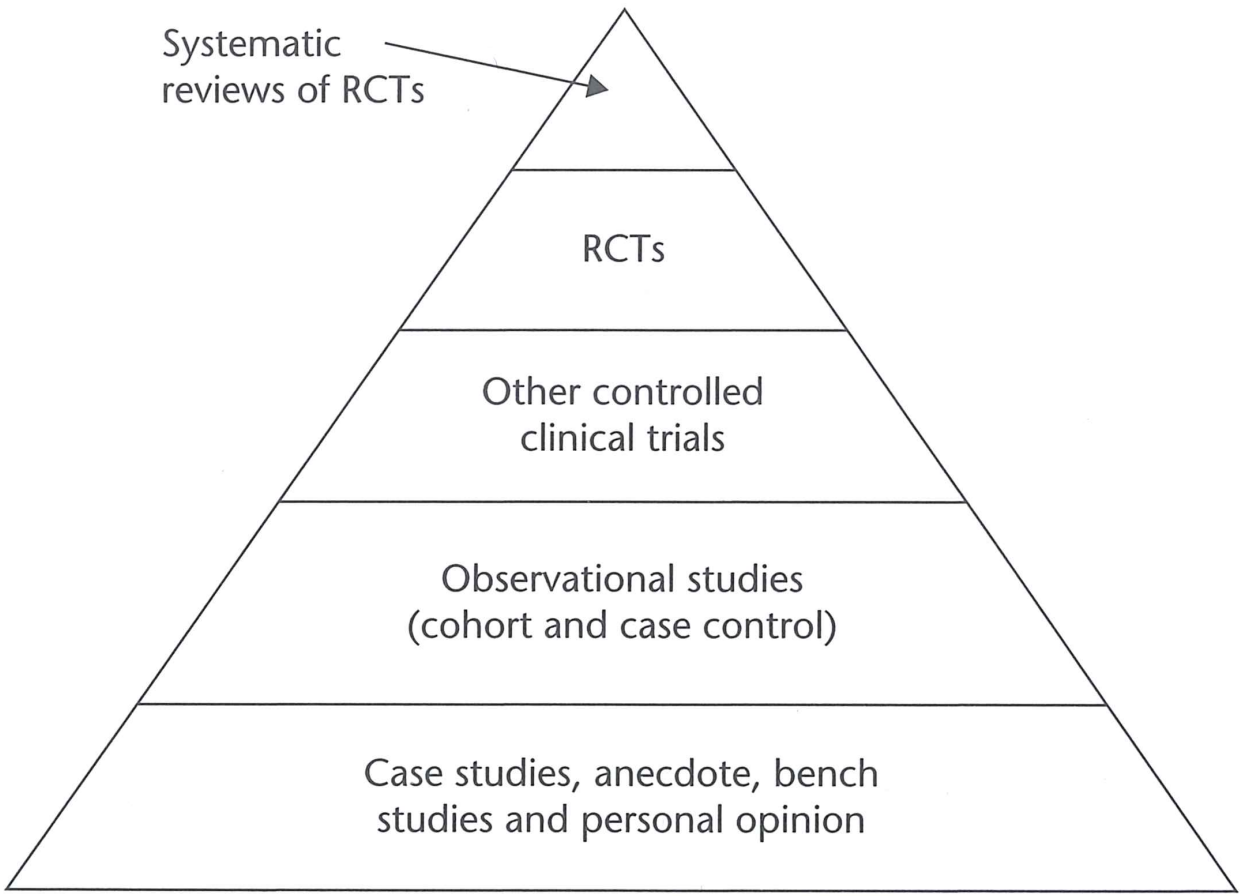

Figure 2.1 A simple hierarchy of evidence for assessing the quality of trial design in therapy studies.

[1] Greenhalgh (2010, p. 18)

CEBM's levels of evidence for intervention studies[2]

1a Systematic reviews (SR) & meta-analyses (with homogeneity) of RCTs

1b Individual RCT (with narrow confidence interval)

2a SR (with homogeneity) of cohort studies

2b Individual cohort study (including low quality RCT)

3a SR (with homogeneity) of case-control studies

3b Individual case-control studies

4 Case-series (and poor quality cohort and case-control studies)

5 Expert opinion without explicit critical appraisal; bench research

[2] Oxford Centre for Evidence-Based Medicine, 2009

Evidence hierarchies for other study types

For evidence hierarchies relevant to studies related to prognosis, diagnosis, and economic and decision analyses, see the CEBM's website: `http://www.cebm.net/index.aspx?o=1025`

RCTs

How effective is this for improving my fitness level?

https://support.apple.com/en-hk/HT204517

Randomised controlled trials (RCTs)[3]

- Two (or more) treatment conditions are compared by randomly allocating participants to groups and controlling for known sources of **study bias**.
- Designed to investigate:
  - Treatment vs. no-treatment (control group)
  - Treatment 1 vs Treatment 2
  - Several treatments
- Level 1b of the Oxford evidence hierarchy

[3] With thanks to David Howard, Newcastle University, UK

RCTs

- Designed to establish the average efficacy of a treatment and learn about frequently occurring side-effects.
- Treatment effects may be small and therefore undetectable, except when studied systematically on a large sample.
- Treatment effects may vary between people, making inferences from single case studies unreliable.

RCTs

- Designed to control for change due to:
  - Spontaneous recovery unrelated to treatment;
  - Maturation, in the case of children;
  - Other, unexpected things.

Essential features of RCTs

- Study participants should be **randomly allocated** to treatment groups.
  - Neither research staff nor participants should be able to influence who gets what treatment.
  - This is controlled by **blinding**.
  - This reduces bias by equalising other factors that have not been accounted for in the design of the study.
- The population from which the sample is drawn should be clearly defined.
  - Results only **generalise** to people from the same population, receiving the same treatment.

Essentials features of RCTs

- The treatments should be clearly defined (e.g., "manualized").
  - This enables others to use the same treatment if it is effective.
  - Sometimes available on a website; e.g. Lidcombe programme manual[4]
- The outcome measures should be clearly defined and relevant to the kinds of benefits anticipated.
  - They should show what kind of benefit and the degree of benefit that can be expected from the intervention.

[4] https://sydney.edu.au/health-sciences/asrc/docs/lp_treatment_guide_2016.pdf

Blinding

- An important study design feature to control bias.
- Single-blind trial
  - Researcher knows the details of the treatment but the patient does not.
  - Eliminates **placebo effects**, but **observer bias** is possible.
- Double-blind trial
  - Clinician, patient and assessor do not know which group the patient was assigned to.
- Not always possible or practical, especially with behavioural interventions.
- **Blind assessors** are very important and usually possible.

RCT data analysis

- Patients are normally analysed within the group to which they were allocated, regardless of whether they experienced the intended intervention (**intention-to-treat analysis**).
- "Intention-to-treat analyses are pragmatic in that they reflect real-world non-adherence to treatment." [5]
- The analysis is focused on estimating the size of the difference in outcomes between intervention groups (effect size).

[5] http://hbiostat.org/doc/glossary.pdf

Limitations of RCTs

- Exposing patients to an intervention believed to be inferior to current treatment is sometimes considered unethical.
- On the other hand, failure to perform trials may result in harmful (or worthless) treatments being used.
- Once an intervention becomes widespread, it can prove impossible to recruit clinicians who are willing to *experiment* with alternatives.

Further limitations

- RCTs are generally more costly and time consuming than other kinds of studies.
- Is the intervention well enough developed to permit evaluation?
- Is there preliminary evidence that the intervention is likely to be beneficial, from other sources of evidence?
- Is there an appreciation of the size of the treatment effect? (Needed to estimate sample size and justify expense of a trial.)

Benefit of RCTs

Well-designed and executed RCTs are the most rigorous way of determining whether a cause-effect relation exists between treatment and outcome and for assessing the cost-effectiveness of a treatment.

Reporting Standards

Reporting standards

- To promote transparent and complete reporting of RCTs
- CONSORT is evidence-based.
- The CONSORT 2010 statement
  - For authors of RCTs
  - 25-item checklist + flow diagram
  - Chinese translation available[6]
  - `http://www.consort-statement.org`
- The EQUATOR network
  - One-stop website for reporting standards for most kinds of clinical studies, including **qualitative studies**
  - `http://www.equator-network.org`

[6] http://www.consort-statement.org/downloads/translations

Critical Appraisal

Critically appraising evidence

- Just because an RCT has been published doesn't guarantee that it was done well.
- Critical appraisal lets you judge for yourself about a study's quality and its value and relevance to your clinical practice.

Critical appraisal checklists

- For RCTs and many other kinds of clinical studies
- Dollaghan (2007) checklists
  - Appendices A–E (may be photocopied; see p. 152)
  - We'll use these in the group discussions in class.
- Scottish Intercollegiate Guidelines Network (SIGN)
  - `http://www.sign.ac.uk/checklists-and-notes.html`
- Oxford CEBM
  - `http://www.cebm.net/index.aspx?o=1157`

Group discussion

- Break up into your assigned groups.
- Use CATE[7] to critically appraise the research articles you read for today.
- Document on the form *where* you found information in the research article addressing each point.
- After the discussion, you may be asked to summarise several points on CATE.

[7] Dollaghan (2007, p. 153)

References

Dollaghan, C. A. (2007). Appraising diagnostic evidence. In C. A. Dollaghan (Ed.), *The handbook for evidence-based practice in communication disorders* (pp. 81–104). Baltimore, MD: Paul H. Brookes.

Greenhalgh, T. (2010). *How to read a paper: the basics of evidence-based medicine* (4th ed.). Chichester: Wiley-Blackwell BMJ Books.