

Choosing a research protocol

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Research case 1

You want to study if a new drug Obecalp is effective in the management of type II diabetes. What study protocol would you choose?



Randomized controlled trial (RTC)

- For many RTC are considered the “gold standard” in research
 - A treatment or process is compared to a placebo in a randomized controlled fashion that minimizes bias and allows for statistical analysis of results
 - Studies are often-blinded (Patient and in some cases Researcher) do not know who is in active treatment arm



RCT – Limiting factors

- Obtaining adequate patient study groups to obtain meaningful results (options may be to partner, as in clinical trials; research networks)
- Determining inclusion/exclusion criteria
- Ethical considerations – i.e., comparing drug intervention to placebo when an effective treatment already exists (option may be to compare to a “gold standard”)
- Limitations of randomization
- Complexity/Cost



87% OF THE 56% WHO COMPLETED MORE THAN 23% OF THE SURVEY THOUGHT IT WAS A WASTE OF TIME

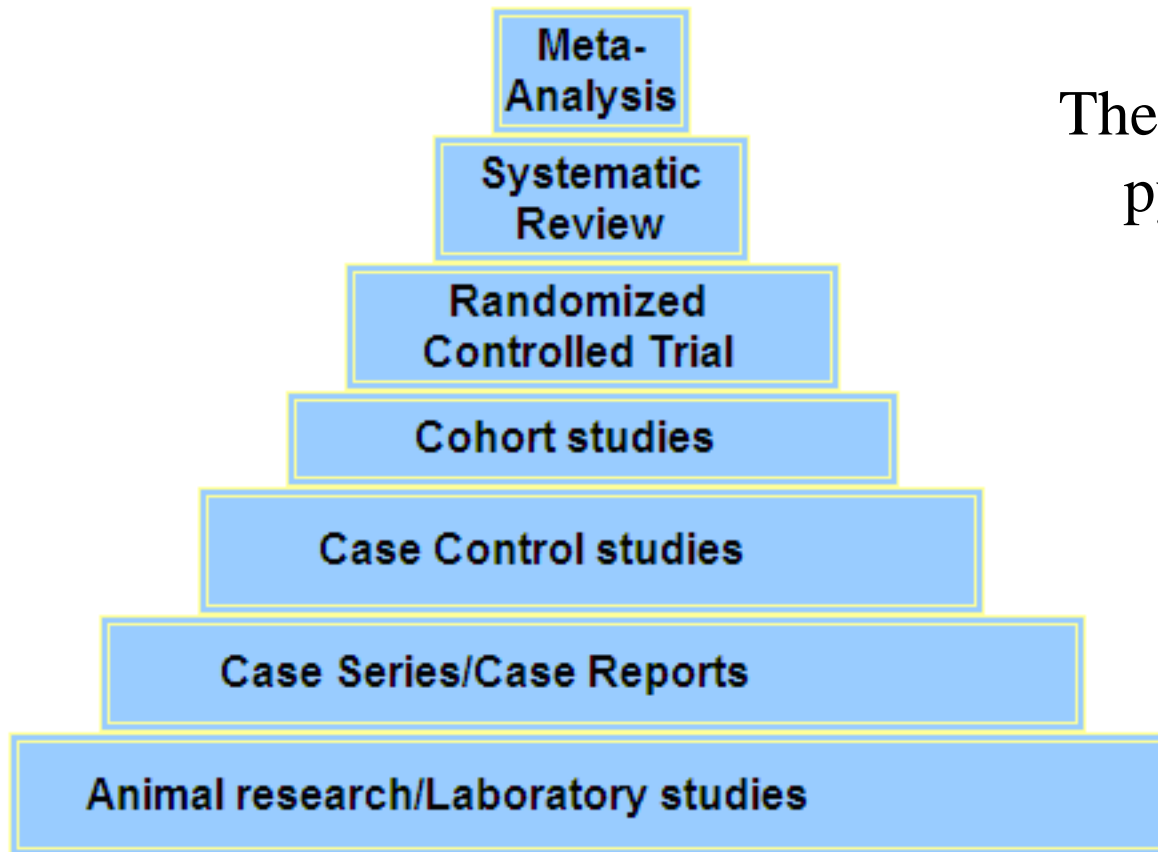


RCT pilot studies – an option

- Pilot studies provide a means to:
 - Perform a smaller study to see if a larger study is needed
 - Test a protocol method to see if needs to be refined before more formal study
 - Provide preliminary data to obtain grant funding



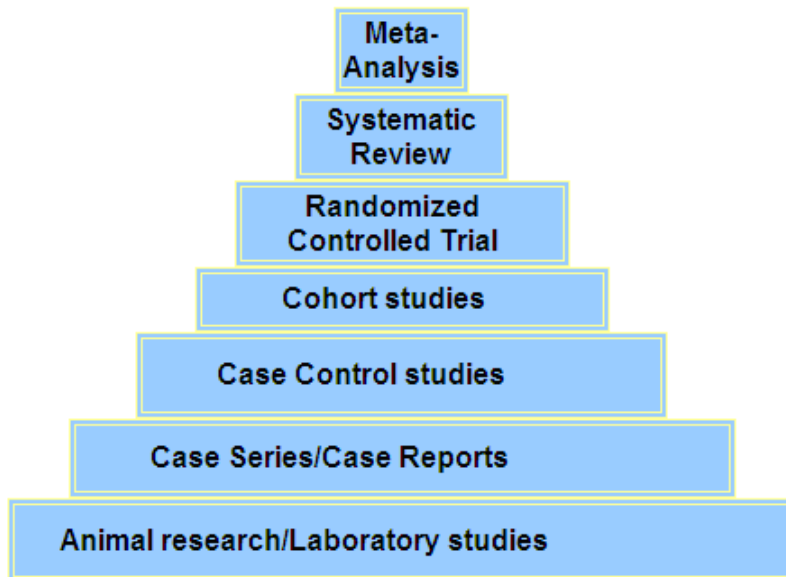
Alternatives to RTC



The evidence pyramid



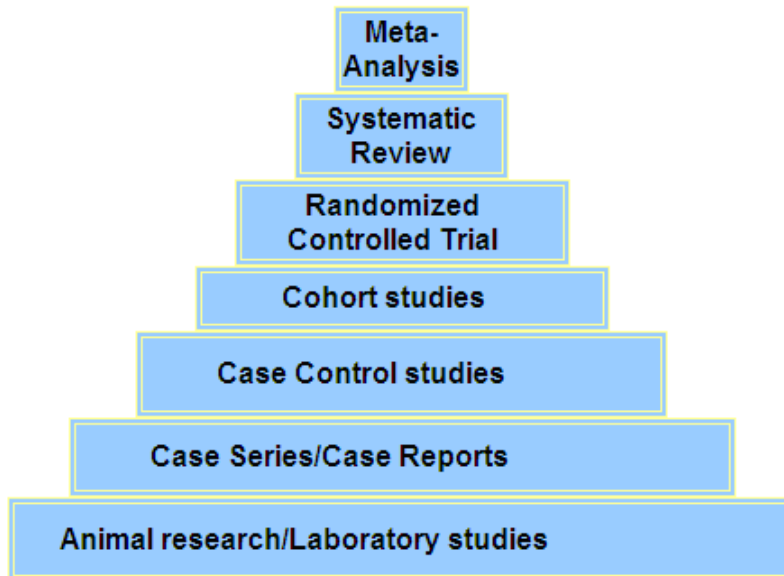
Alternatives to RTC continued



- **A Meta-analysis** will thoroughly examine a number of valid studies on a topic and combine the results using accepted statistical methodology as if they were from one large study. Some clinicians put Meta-analysis at the top of the pyramid because part of the methodology includes critical appraisal of the selected RCTs for analysis.
- **Systematic Reviews** usually focus on a clinical topic and answer a specific question. An extensive literature search is conducted to identify all studies with sound methodology. The studies are reviewed, assessed, and the results summarized according to the predetermined criteria of the review question. *The Cochrane Collaboration* has done a lot of work in the area of systematic reviews.
- **Randomized controlled clinical trials** are carefully planned projects that study the effect of a therapy on real patients. They include methodologies that reduce the potential for bias (randomization and blinding) and that allow for comparison between intervention groups and control groups (no intervention).



Alternatives to RTC continued



- **Cohort Studies** take a large population and follow patients who have a specific condition or receive a particular treatment over time and compare them with another group that has not been affected by the condition or treatment being studied. Cohort studies are observational and not as reliable as randomized controlled studies, since the two groups may differ in ways other than in the variable under study.
- **Case Control Studies** are studies in which patients who already have a specific condition are compared with people who do not. They often rely on medical records and patient recall for data collection. These types of studies are often less reliable than randomized controlled trials and cohort studies because showing a statistical relationship does not mean that one factor necessarily caused the other.
- **Case series** and **Case reports** consist of collections of reports on the treatment of individual patients or a report on a single patient. Because they are reports of cases and use no control groups with which to compare outcomes, they have no statistical validity.



Research Case 1 summary

- Clinical research – goal is to determine efficacy of treatment/intervention. In this case a pilot study was developed to investigate efficacy in a small group and potential issues with recruiting patients for study.



Research Case 2

Despite aggressive educational programs, dedicated diabetic clinics, and expanded clinic hours, the diabetic population of your clinic continues to have markedly elevated HbA1c levels. You want to find out why. What research method(s) would you consider?





Qualitative vs. Quantitative research

Qualitative research is designed to “explore” a problem. Examples include focus groups, Cohort or case studies where observations are made, and open-ended survey questions.

- Help to determine possible issues/concerns
- Challenge is summarizing results – one method is objective coding (grouping results by similar theme)

Quantitative research helps to determine which factors are of greatest importance. results. Examples include survey results.

Can provide insight in frequency/importance of an issue but does not “explore”

Ideal research approach is combining both methods – use small focus groups to determine issues and then survey instrument to quantify responses across an entire study group



Qualitative research – focus groups

- Focus groups may be patients, medical providers, care givers, nurses
- Making focus groups work
 - Create comfortable environment
 - Ask open ended questions (may be facilitated with a hand-out or PowerPoint slide)
 - Encourage everyone to respond
 - Record session



Qualitative research – focus groups (continued)

- To analyze data, “Code” or categorize themes from responses
- May benefit from outside, non-biased coders



"I thought you did fine, but I'm afraid you didn't test well with the focus group."




Research Case 2 summary

- A focus group discussion revealed the following challenges to good diabetic control:
 - Cost to of medications
 - Diet recommendations do not fit cultural preferences
 - Patients did not understand what HbA1c test meant
 - Patients thought medications ‘cured’ diabetes so they did not need to take medications once blood sugars normal
- A survey of the entire diabetic population found that the “cure’ issue was the most common challenge to good control – educational intervention developed.

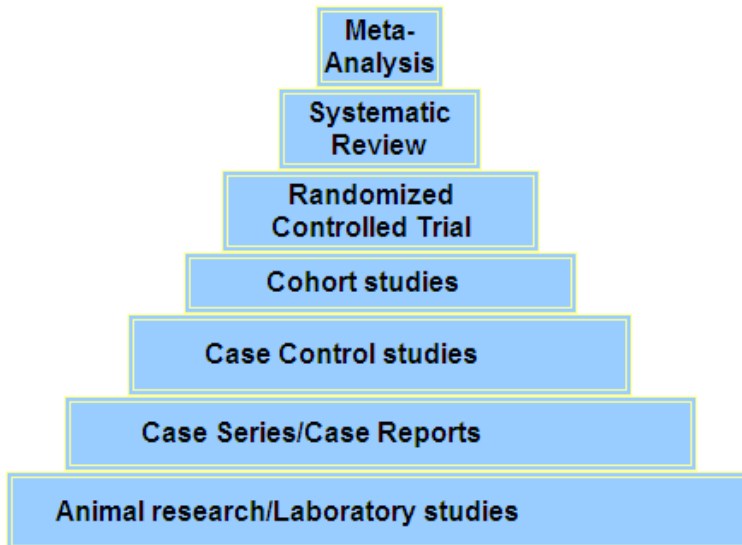


Research Case 3

You wish to determine whether a diabetic education classroom experience or a web-based educational experience is more effective in reaching you diabetic population. What research method(s) would you consider?



Research case 3 – what protocol would you suggest



- Possible study protocols
 - RCT
 - Cohort study
 - Case control study



The question is not just “what type study”
but “what **OUTCOME** do you wish to
measure?”

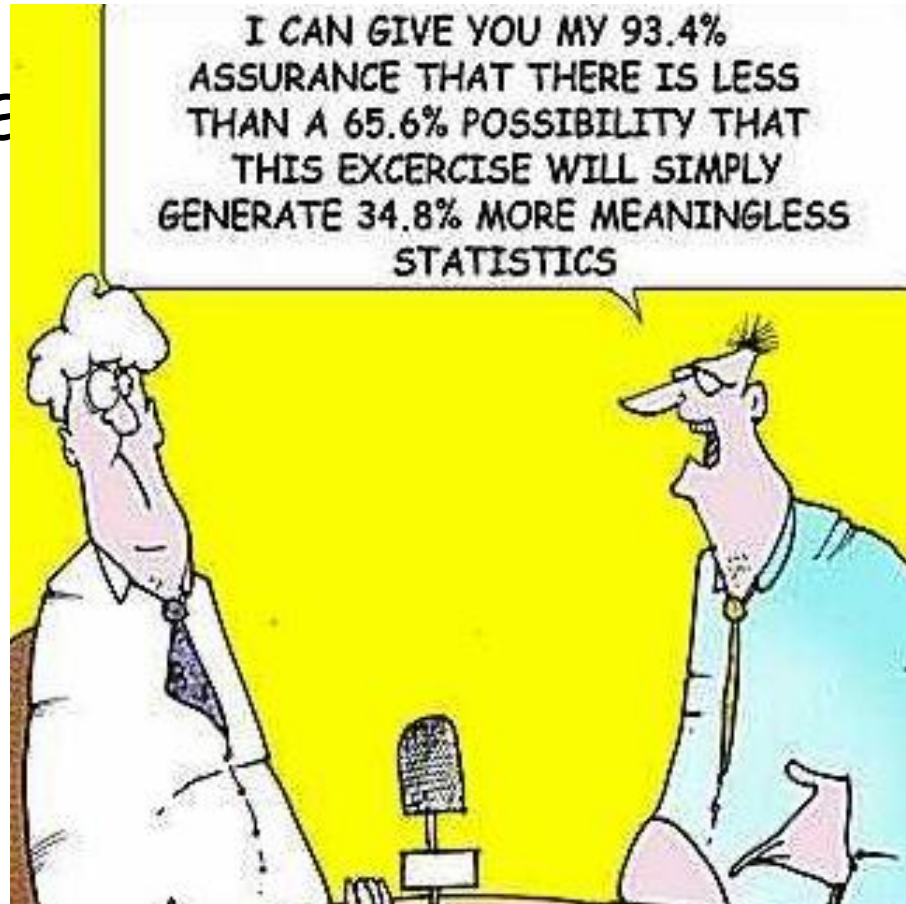
You could compare:

- HbA1c levels in both groups
- Patient satisfaction
- Assessment of knowledge gained



The next question is 'How should I analyze results?'

Answer – ta





Research case 3 summary

Two study populations identified (in randomized fashion) ANOVA analysis of HbA1C levels between the 2 study groups planned.



Research protocol algorithm

Is purpose of research to “explore” new ideas/concepts

Yes

Consider qualitative method (focus group, cohort observation) with qualitative follow-up

No

Can research question be answered using existing data?

Yes

Consider met-analysis or systematic review

No

Do you resources to perform randomized control study (RCT)?

Yes

Determine outcome (s) to be measured and work with statistician to develop protocol

No

Do you have resources to perform pilot RCT ?

Yes

Determine goal of pilot study (i.e., to test protocol vs. determining pilot data).



A few words about funding

- Pilot data ALWAYS helps
- Look for funding from organizations that wish to answer your research question
- Look locally first (including to OPTI-West, host hospital) many organizations provide funding for pilot or small projects
- Build research “C.V.” – funding leads to more funding