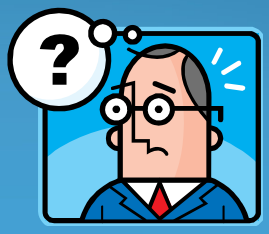
Writing a Pilot or Feasibility Study

"You never test the depth of a river with both feet"



Pilot and feasibility studies are not the same thing.

Questions around both revolve around "Can this study work?"

NIH mechanisms:

UH3/UG3, R03 sometimes, first phase of RCT, sometimes under special PA

Often milestone driven

What is a pilot study?

- A miniature version of the main study run to determine if the components and processes can all work together to generate results
- A shake-down cruise of the main study
- Focused on procedures: recruitment, randomization, follow-up visits
- Particularly important for complex interventions

What a pilot study is not.

- It is not a study you undertake when you have no hypothesis.
- It is not a study that has a sample size that is too small to matter.
- It is not a small, underpowered clinical trial…"pilot" does not make it ethical.
- It is not a study that has no follow-up planned.
- It is very difficult to be funded for a pilot as it is an **expected** component of any study implementation.

Types of Pilot Studies

- Internal pilot = data will be incorporated
- External pilot = data will not be used
- Can help you to recalculate power, sample size

What is a feasibility study?

- Components of research done prior to the main study to answer the question, "Can this study be done?" Is it feasible?
- Undertaken ***before the real work*** to determine whether to proceed with a project and to decide the best approaches to adopt
- Important in reducing uncertainties for the main study
- This work can provide reassurance to reviewers that the work has been thoroughly thought through

Thinking about feasibility…

- Acceptability
- Demand
- Implementation
- Practicality
- Adaptation
- Integration (system changes)
- Expansion (intervention in a different disease focus)
- Limited efficacy testing

Common to use a mixed-methods approach.

Feasibility Study

The endpoints for a feasibility study are factors that affect successful trial conduct, not measures of treatment effect or safety.

- What do we need to know that we currently don't in order to make the main study a success?
- What could go wrong in the main study?
- This is **not** a "study in miniature"

Writing a feasibility study

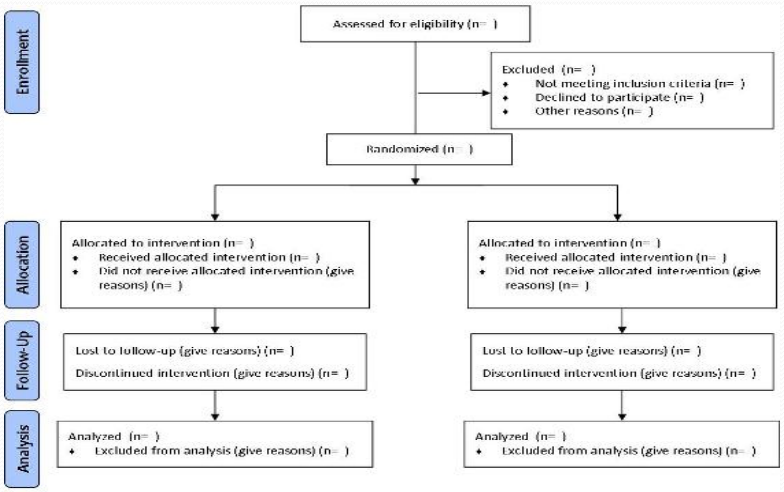
- What do you need to know to propose the "real" study you want to conduct?
  - Increase reviewer's confidence
  - Generate confidence about your ability to do this study
  - Generate data for sample size and power
- A description of a clear route of progression to the main study is paramount
- Can include hypothesis testing around feasibility
- Specific aims need to clearly delineate the steps you are undertaking, why, and what success means
- Power calculations are usually unnecessary
  - However, the sample size should be adequate to estimate parameters such as recruitment rate and sample variability

Feasibility Study Questions

Study Participants

- How easy are they to identify?
- Can you demonstrate you have access to sufficient subjects?
- Proportion of the available population that are eligible
- How diverse/similar are the subjects?
- How realistic are the eligibility criteria?
  - Is it obvious who meets and who does not meet the eligibility requirements?
- How willing would they be to be recruited and/or randomized?
- What is the refusal rate?

Think about the CONSORT diagram….



Feasibility Study Questions

Study Sites

- In recruiting sites, what makes the site useful? Number of patients available? Diversity? Age? Community sites?
- How suitable are the investigators in terms of qualifications, experience, probity?
- What facilities and staff do they have at their center that help the study?
- How well did they recruit patients as an indicator of what might happen in the main study?
- How willing are they to recruit or randomize patients when faced with alternatives?

Feasibility Study Questions

Study Processes

- Retention rates
- (Non)compliance or adherence rates
- Understanding of study questionnaires or data collection tools:

  - Do subjects provide no answer, multiple answers, qualified answers, or unanticipated answers to study questions

  - Run a cognitive test of selected instruments

A chance to involve the community

- Stakeholder engagement!
- Community of clinics/practices
- Community of similar patients and their families
- Can help to determine:
  - How the intervention will be implemented
  - How frequently patients are willing to return for follow-up
  - How best to collect information…internet, SMS, mail
  - Are participants willing and able to answer the questions and complete the procedures?

Feasibility Study Questions

Power and Sample Size

- How frequently does the outcome occur in the population you expect to recruit from? (baseline)
- Expected range of the outcome, sample variance
- What is the variability of other important variables?

Questions more specific to pilot studies

- Determining capacity:
  - Will the study participants overload your phone lines or overflow your staff's capacity to recruit and perform study visits?
- Determining process time
  - How much time does a study visit take?
  - Is the equipment readily available?
  - What backup is available?
- Are there problems collecting and entering data?
- Determining center willingness and capacity
  - Do the centers do what they committed to doing?
  - Do investigators have the time to perform the tasks they committed to doing?
  - Are there any capacity issues at each participating centre?

Critical points that will not come from pilot or feasibility

Scientific:

- Is it safe to use the study drug/intervention?
- What is the safe dose level?
- Do patients respond to the drug? (maybe not best question)
- What is the estimate of the treatment effect?
- What is the estimate of the variance of the treatment effect?

Example of feasibility SA

- Determine attitudes around implementation of a daily anaerobic exercise program for women over 60 with T2DM. Barriers, facilitators
- Explore clinic processes in practices in rural Oregon to determine the optimal way to approach their patients to participate in research.
- Determine the willingness of women >60 years with T2DM to participate in the proposed study. What are the barriers and facilitators?
  - feasible if >70% of eligible participants agree to participate.
- Identify, recruit, and consent an eligible cohort and determine the proportion who are adherent to the study protocol over a short period of time.
  - Example: Of those participants who provide consent, at least 80% will complete anaerobic exercise for at least 45 minutes duration at least 3 days a week.
- Determine the reliability and accuracy of the primary outcome measurement as compared to the reference standard.

Writing the study outcomes

- State the criteria for success *a priori* – benchmarks for success
  - Stop
  - Continue but modify protocol
  - Continue but monitor
  - Continue, no change