

Essentials of Pilot Study Design and Conduct

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Rationale

- High quality pilot data are essential for developing research and external funding
- Yet, formal training in the knowledge and skills necessary for performing high quality pilot studies is rarely available

Objectives

- To define pilot studies
- To understand the challenges in writing a pilot study protocol
- To understand the challenges in conducting a pilot study
- To list funding opportunities for pilot studies

Pilot Study Definition

- A preliminary investigation intended to collect data to prepare for a larger, more definitive study.
- Generally small in size, scope, duration and budget



A Really Good Reason to Do a Pilot Study

- A pilot study allows you to know what things go wrong so you can fix them before you start the large study.



Challenges in Writing and Conducting a Pilot Study Protocol



Challenges in Writing a Pilot Study



Proposal Length

- Short - most pilot proposals a few pages
 - Requires concise writing
 - Good resource: Gopen, G. D.; Swan, J. A. The Science of Scientific Writing. *American Scientist* 1990, 78 (6), 550–8.

Specific Aims

- Not definitive hypothesis testing
 - Still requires well defined, purposeful objective(s)
 - Embedded in a larger good idea or leading to a good, innovative idea/project
 - Necessarily limited in number and scope - Keep these focused and feasible

Significance

- Emphasize the importance of area of inquiry and potential product in small space
 - Scholarship has to be sharp and to the point, the key articles by the key people
 - Rationale for the pilot clearly defined
 - Theoretical model may or may not be necessary

Should Pilot Data Be Included in a Pilot Study Application?

- Yes, if it makes the case for the importance and sound conduct of the pilot and for the particular funding mechanism – e.g., R03, R21

Sample Selection

- By definition, working with small samples
 - Use as rigorous a strategy as possible but recognize the risk of less representative samples
 - Try to make sure that your pilot subjects cover the entire range of subjects in your full study
 - Do not slap on the label of pilot study when your sample size is too small

Approach

- Feasibility
 - Beware of the tendency to propose or do too much
- Recruitment and retention
 - Propose a realistic recruitment and retention plan
 - Reviewers know that recruitment difficulties extend to pilot studies

Sample Size

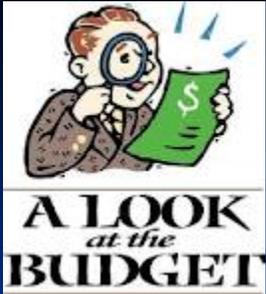
- How Many Subjects for My Pilot Study?
 - Depends on the objective of the study
 - Some pilots don't require formal sample size calculations
 - Enough observations to provide useful information
 - 95% Confidence interval approach if you know target for success (e.g. 70% of patients are able to complete the form)

Cautions When Determining Effect Size for Powering Larger Study

- Design of the pilot is different from that actually employed in the trial (leading to different effect sizes)
- Pilot is run by the interested, skilled, and dedicated researcher while the follow-up confirmatory experiment is run by less interested, skilled, and dedicated personnel (leading to reduced effect sizes in the followup study)

Analysis

- Be clear about how the data will be interpreted and utilized
- Analyses mainly descriptive although p values or confidence intervals appropriate in some situations
- Treat results as preliminary and interpret with caution



Budget



- Usually for specific expertise (data management, statistics, consultant), supplies, part of study assistant effort
- Usually not for investigator salaries, full time study coordinators, equipment, travel
- Pilots help understand resource requirements in full study

Potential for Extramural Support

- Does the project have a high likelihood of leading to future extramural, larger grant support?
- Be explicit about how/where pilot results will fit with larger grant- place the pilot study in the context of the full-blown study.

Challenges in Conducting a Pilot Study



Challenges in Conducting a Pilot Study

- Short time frame
- IRB approval
 - A pilot takes as long as a large study
- Subject recruitment and retention
 - Don't let small number of subjects needed deceive you
- Personnel problems
 - Research assistant gets sick, co-investigator loses interest, statistician moves to another institution

Challenges in Conducting a Pilot Study

- Short time frame
- Competing demands on your time
 - Teaching load changes, more clinic or rounding time
- Supply chain problems
 - Animals, reagents, databases hard to get
- Data inconclusive
 - Uninformative numbers

Challenges in Conducting a Pilot Study

- Sort time frame
- Changes in laboratory or clinical practice
 - Affects recruitment, measures, interventions
- Equipment breakdown
 - Flow cytometer, multiplex assay system not working?
- "I never thought about that!"

Sources of Funding for Pilot Studies

- NIA early stage research mechanisms
- R03s – small grants (50k a year, two years)
 - <http://www.nia.nih.gov/research/dea/r03-small-research-grants>
- R21s – exploratory/developmental grants (\$275k over two years)
 - Exploratory/Developmental Research Grant Award
 - <http://grants.nih.gov/grants/funding/r21.htm>
- NIA Center Programs
 - Older Americans Independence Centers
 - Demography Centers
 - Resource Centers on Minority Aging Research

Sources of Funding for Pilot Studies

- K24 (Mid-Career award in Patient-Oriented research)
 - Up to \$50,000 a year can be used to provide pilot funding
- K07 (Academic Leadership Award)
 - A portion of the award may be used for pilot funding
- NIH Clinical and Translational Science Awards (CTSA) have local small grant or pilot study mechanisms

Sources of Funding for Pilot Studies

- AHRQ Small Research Grant Program (R03)
 - Priority areas include translating research into practice, patient safety and quality, patient centered care, payment and organization
 - <http://grants.nih.gov/grants/guide/pa-files/PAR-10-168.html>
- AFAR Research Grants
 - Up to \$100,000 for a one- to two-year award to junior faculty, broad range of biomedical, clinical topics
 - <http://www.afar.org/research/funding/>
- Robert Wood Johnson Foundation
 - multiple grants in \$50,000-100,000/year on wide range of health topics, some applicable to geriatrics <http://www.rwjf.org/grants/>
- VA Research - VA Research Foundation small grants

Sources of Funding for Pilot Studies

- Specialty Associations
 - American Heart Association Affiliate grant Programs
 - American Diabetes Association
- State, Local Community or Institutional Small Grants