Clin-STAR
Clinician - Scientists Transdisciplinary Aging Research Coordinating Center

CLIN-STAR WEBINAR

Clin-STAR
Clinician - Scientists Transdisciplinary Aging Research Coordinating Center

The Art and Science of the Pilot Study: Challenges and Solutions

October 16, 2025
4-5pm ET

Speakers

Kenneth Schmader, MD
Duke University School of Medicine

Carl F. Pieper, DrPH
Duke University School of Medicine

NIH National Institute on Aging | afar american federation for aging research

The Art and Science of the Pilot Study: Challenges and Solutions

Kenneth Schmader, MD
Director, Pepper Older Americans Independence Center (OAIC)
Carl F. Pieper, DrPH
Co-Lead, Analysis Core, Pepper OAIC
Duke University School of Medicine
Durham, NC

Rationale

- High quality pilot study data are essential for conducting definitive studies and for obtaining external funding
- Yet, little training in the knowledge and skills necessary for performing high quality pilot studies

Objectives

- To describe challenges and solutions in the design of a pilot study protocol
- To describe challenges and solutions in the conduct of a pilot study
- To list publication and funding opportunities for pilot studies

Pilot Study Definition

- A preliminary, small study preparatory to a larger, definitive study
- A small-scale test of the methods and procedures to be used on a larger scale

Kistin C et al. JAMA 2015;314:1561-1562
Leon AC et al. J Psychiatr Res 2011;45:626-629

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 and Solutions in the Design of a Pilot Study

Specific Aims

Challenges

- Too many
- Too broad
- Hypothesis testing

Solutions

- Fewer in number and scope – focused and feasible
- Usually not definitive hypothesis testing
- Pilots should not test Ho, if you reject Ho, then why move to larger study?
- Engage statistician early in proposal as collaborator

Common Aims of Pilot Studies

- Acceptability, fidelity, feasibility
- Feasibility of recruitment, randomization, retention
- Adherence
- Integrity of study protocol
- Testing of measures, data collection
- Assessment procedures
- Data management

Lancaster GA et al,. J Eval Clin Pract 2004: 10; 307-12.
Thabane et al. BMC Medical Research Methodology 2010, 10:1
Leon AC et al. J Psychiatr Res 2011;45:626-629

Sample Size

How Many Subjects for My Pilot Study?

Challenges

- Unclear rationale for proposed number of subjects
- Uncertainty about appropriate number

Solutions

- Many pilots don't require formal sample size calculations
- Enough observations to provide useful information
- 95% Confidence interval approach if you know target for success

Kraemer et al. Arch Gen Psychiatry 2006;63:484-489
Thabane et al. BMC Medical Research Methodology 2010:10:1

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)
- Make sure you know what outcome will be studied. Don’t pick the winner

Kraemer HC, et al. Arch Gen Psych. 2006;63:484-489.

Sample Selection, Recruitment and Retention

Challenges

- Enrolling representative sample
- No or poorly described recruitment and retention plan

Solutions

- Attempt enrollment of range of subjects anticipated in larger study
- Propose a realistic recruitment and retention plan

Leon AC et al. J Psychiatr Res 2011;45:626-629

Analysis

Challenges

- Lack of clarity about how data analyzed
- No metrics
- Beware of p-values & tests of significance, hypothesis testing

Solutions

- Clear analysis plan – include statistician at beginning
- Add and define metrics
- Analyses mainly descriptive
- No P-value test of your H0:
- Treat results as preliminary and interpret with caution

Moore, C.G., et al. Clinical and Translational Science, 2011;4: 332-337

Add & Define Metrics

- Example:
  - Recruit & consent 50 participants
  - Retain 90% of the participants over 12 months
  - 80% of participants will attend 90% of the prescribed sessions.
  - 80% of the participants will find the intervention as acceptable

Test Aims

1. Our recruitment goal was 50 subjects, we consented 52
2. 93% of those randomized, were retained.
3. The average participant attended 90% of the sessions, and 95% attended 80 or more of the prescribed sessions
4. 93% of the subjects agreed that the protocol was helpful.

Relation of Pilot Study to Larger Definitive Study

Challenges

- Not clear how pilot results will fit in the larger grant
- Lack of follow-up plan
- Doubt that pilot results will lead to larger grant

Solutions

- Be explicit about how/where pilot results will fit with larger grant
- Place the pilot study in the context of the full study
- Convince reviewers that the pilot has a high likelihood of leading to future extramural, larger grant support

Moore, C.G., et al. Clinical and Translational Science, 2011;4: 332-337

Budget

Challenges

- Small amount of money
- Usually not enough for substantial investigator, statistician, data management, study coordinator effort

Solutions

- Usually for specific expertise, supplies, part of study assistant effort
- Leverage existing resources at your institution
- Pilots help understand resource requirements in full study

Challenges and Solutions in the Conduct of 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 deceive you
- Personnel problems
  - Research assistant gets sick, co-investigator loses interest, statistician moves to another institution, personnel costs
  - Budget!

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

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?
- Blind spots
  - "I never thought about that!"

Pilot and Feasibility Studies Journal

- Aims and Scope
  - *Pilot and Feasibility Studies* encompasses all aspects of the design, conduct and reporting of pilot and feasibility studies in biomedicine. The journal publishes research articles that are intended to directly influence future clinical trials or large scale observational studies, as well as protocols, commentaries and methodology articles. The journal also ensures that the results of all well-conducted, peer-reviewed, pilot and feasibility studies are published, regardless of outcome or significance of findings.
  - https://link.springer.com/journal/40814
  - Home | Pilot and Feasibility Studies

Sources of Funding for Pilot Studies

- NIA early stage research mechanisms
- R03s – Small Research Grants (50k direct costs a year, two years)
  - http://www.nia.nih.gov/research/dea/r03-small-research-grants
  - Grants for Early Medical/Surgical Specialists' Transition to Aging Research (GEMSSTAR) | National Institute on Aging (nih.gov)

Sources of Funding for Pilot Studies

- NIA Center Programs
  - Pepper Older Americans Independence Centers
  - Nathan Shock Centers
  - Alzheimer's Disease Research Center
  - Centers on the Demography and Economics of Aging
  - Roybal Centers for Translational Research in the Behavioral and Social Sciences of Aging
  - Resource Centers on Minority Aging Research

Sources of Funding for Pilot Studies

- Research Collaborating Centers Network
  - https://www.rccn-aging.org/rccn-pilot-awards-request-for-applications
- Clinician-Scientists Transdisciplinary Aging Research Coordinating Center (Clin-STAR)
  - Clin-STAR Pilot Grants
- US Deprescribing Research Network
  - Home - US Deprescribing Research Network

Sources of Funding for Pilot Studies

- ASCENT (Advancing the Science of Palliative Care Across the Lifespan)
  - Pilot and Exploratory Studies Award | ASCENT Consortium
- Artificial Intelligence and Technology Collaboratories (AITC) for Aging Research
  - https://www.a2collective.ai/pilotawards

Sources of Funding for Pilot Studies

- K24 (Mid-Career award Patient-Oriented research)
  - Award has funds for research expenses and/or statistical services for mentees
- K07 (Academic Leadership Award)
  - A substantial portion of the $100,000 a year may be used for pilot funding
- NIH Clinical and Translational Science Awards (CTSA)
  - local small grant or pilot study mechanisms
- Institutional CDAs
  - KL2 or K12 provide funding for pilot projects to support early career investigators

Sources of Funding for Pilot Studies

- AHRQ Small Research Grant Program (R03)
  - PA-24-155: AHRQ Small Health Services Research Grant Program (R03)
- AFAR Research Grants
  - Funding Opportunities - American Federation for Aging Research (afar.org)
- Robert Wood Johnson Foundation
  - Funding and Research Opportunities (rwjf.org)

Sources of Funding for Pilot Studies

- Specialty Associations
  - American Heart Association Grant Programs
    - 2026 Innovative Project Award - Professional Heart Daily | American Heart Association
  - American Diabetes Association
    - Current Funding Opportunities | American Diabetes Association
  - HIV and Aging Consortium – CFARs/OAICs/HARC
    - HIV and Aging Research Consortium
  - Cancer and Aging Research Group (CARG)
    - CARinG Pilot Grants – Cancer and Aging Research Group
- State, Local Community or Institutional Small Grants

Selected References

- Eldridge SM, et al. Defining feasibility and pilot studies in preparation for randomised controlled trials: Development of a conceptual framework. PLoS One. 2016;11(3):e0150205.
- Eldridge SM et al. CONSORT 2010 statement: extension to randomized pilot and feasibility trials. BMJ 2016;355. http://www.equator-network.org/reporting-guidelines/consort-2010-statement-extension-to-randomized-pilot-and-feasibility-trials/
- Kraemer H et al. Caution Regarding the Use of Pilot Studies to Guide Power Calculations for Study Proposals. Arch Gen Psychiatry 2006;63:484-489
- Kistin C et al. Pilot Studies: A Critical but Potentially Misused Component of Interventional Research. JAMA 2015;314:1561-1562
- Lancaster GA et al. Design and analysis of pilot studies: recommendations for good practice. J Eval Clin Pract 2004: 10; 307-12
- Lancaster, G.A., Thabane, L. Guidelines for reporting non-randomised pilot and feasibility studies. *Pilot Feasibility Stud* **5**, 114 (2019). https://doi.org/10.1186/s40814-019-0499-1
- Leon AC et al. The role and interpretation of pilot studies in clinical research J Psychiatr Res 2011;45:626-629
- Moore, C.G., et al. Recommendations for Planning Pilot Studies in Clinical and Translational Research. Clinical and Translational Science, 2011;4: 332-337. https://doi.org/10.1111/j.1752-8062.2011.00347.x
- National Center for Complementary and Integrative Health, NIH. Pilot Studies: Common Uses and Misuses. Pilot Studies: Common Uses and Misuses | NCCIH
- Thabane et al. A tutorial on pilot studies: the what, why and how. BMC Medical Research Methodology 2010, 10:1