# BUILDING A SUCCESSFUL PILOT STUDY: DESIGN AND METHODS CONSIDERATIONS

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# PRIMARY GOAL: FEASIBILITY

- One definition of a pilot study:

  "Preparatory studies designed to test the performance characteristics and capabilities of study designs, measures, procedures, recruitment criteria, and operational strategies that are under consideration for use in a subsequent, often larger, study."

- Highly recommended reference:

  Moore CG, Carter RE, Nietert PJ, Stewart PW. Recommendations for planning pilot studies in clinical and translational research. Clin Transl Sci. 2011 Oct;4(5):332-7. doi: 10.1111/j.1752-8062.2011.00347.x.

# WHAT OFTEN HAPPENS WITH PILOTS

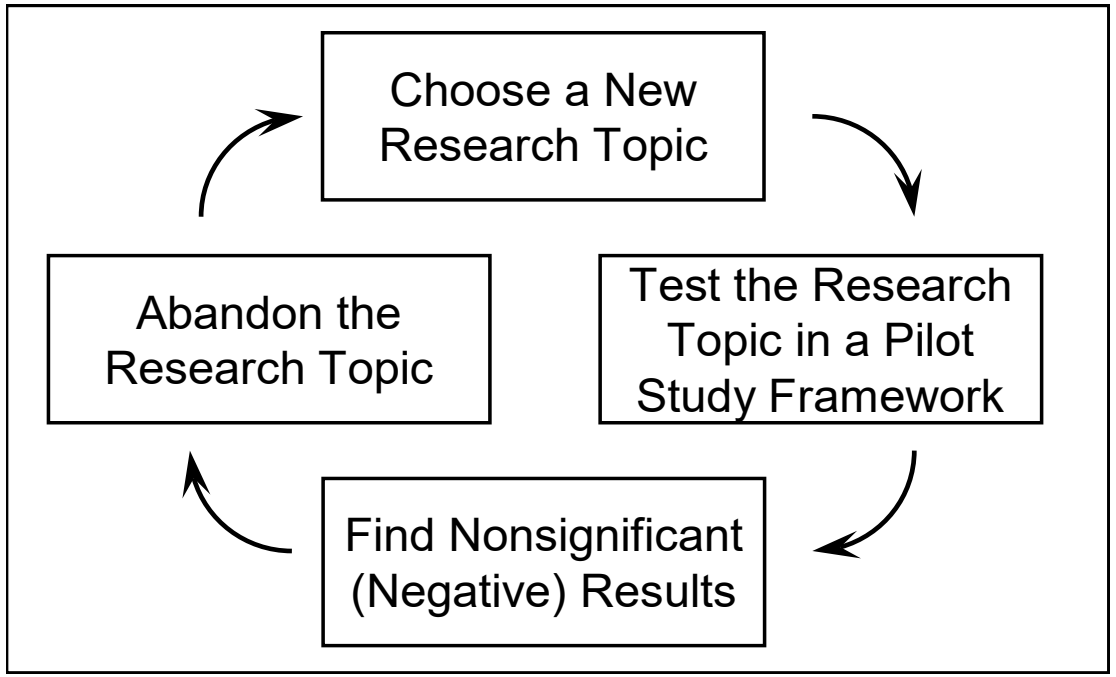
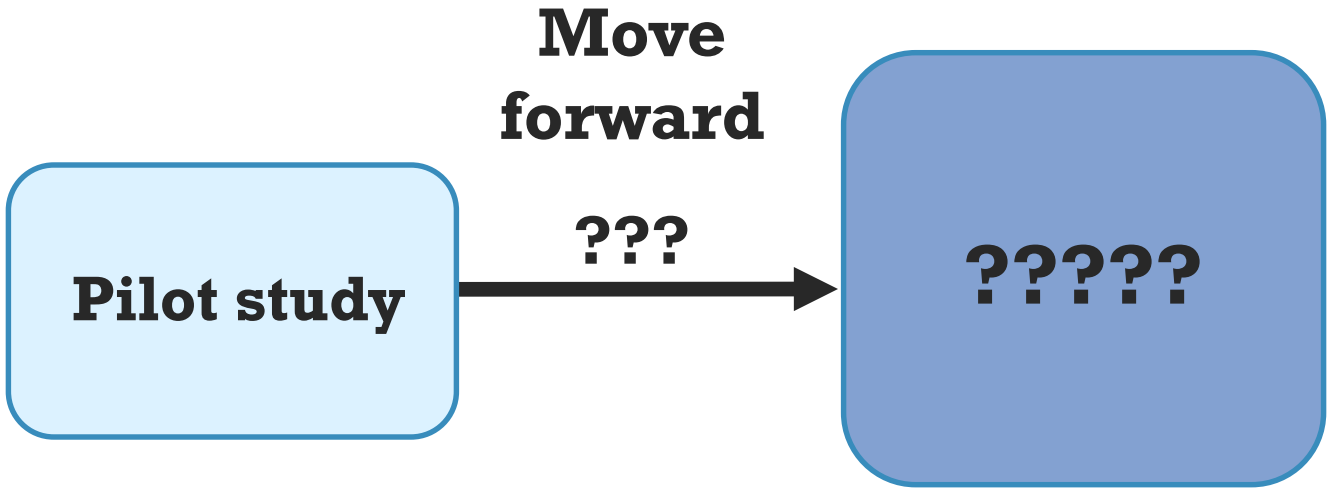



Figure from: Moore CG, Carter RE, Nietert PJ, Stewart PW. Recommendations for planning pilot studies in clinical and translational research. Clin Transl Sci. 2011 Oct;4(5):332-7. doi: 10.1111/j.1752-8062.2011.00347.x.

# GOAL: KEEP THE NEXT STUDY IN MIND

- Pilot study aims and methods should align with the goals of the subsequent study

- Aims of a pilot can range from evaluating feasibility of the protocol to investigating potential mechanisms of efficacy for a new intervention



# PILOT STUDY OBJECTIVES

- Contribute to the development and design of future (larger) studies by:
  - Refining the research hypotheses
  - Identifying barriers to successful study completion
  - Evaluating acceptability of methods and instruments to participants
  - Estimating the time required for study participation

# PILOT STUDY OBJECTIVES

- Contribute to the development and design of future (larger) studies by:
    - Providing estimates of missing data and dropout
    - Estimating rates and variability in outcomes
    - Testing mechanistic efficacy / ‘proof of concept’

# DESIGN OF A PILOT STUDY

- What is the larger study?
  - Population and design are often the same
    - Obtain relevant estimates
    - Demonstrate feasibility
      - Ex: Will participants be willing to be randomized?

- What is being tested in the pilot?
    - Study design
    - Measures & procedures

# EXTERNAL VS. INTERNAL PILOTS

- External Pilot
  - Separate from larger trial

- Internal Pilot
  - Interim analysis to assess sample size assumptions

# PILOT STUDY OUTCOMES

- How are outcomes operationalized?
  - Feasibility
    - Recruitment
    - Implementation
    - Acceptability
  - Variability
  - Response rates

# PILOT FEASIBILITY OUTCOMES

- Screening — Number screened per month
- Recruitment — Number enrolled per month
- Randomization — Proportion screened eligible who enroll
- Retention — Treatment-specific retention rates

Leon, Davis, Kraemer. The role and interpretation of pilot studies in clinical research. Journal of Psychiatric Research 2011; 45: 626-629.

# PILOT FEASIBILITY OUTCOMES

| | |
|---|---|
| • Treatment adherence | Rates of adherence to protocol for each intervention |
| • Treatment fidelity | Fidelity rates per unit monitored |
| • Assessment process | Proportion of planned ratings that are completed; duration of assessment visit |

Leon, Davis, Kraemer. The role and interpretation of pilot studies in clinical research. Journal of Psychiatric Research 2011; 45: 626-629.

# DO WE NEED A CONTROL GROUP?

- Guidance is not consistent and depends on context
- However:
  - Inclusion of a control group allows for a more realistic examination of recruitment, randomization, implementation of interventions, blinded assessment procedures, and retention in blinded interventions

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# DATA MANAGEMENT PLANS

- Pilot Studies **should have:**
  - Good data management (e.g., REDCap not Excel)
    - Excellent time to develop/test data collection process for the larger study

# DATA ANALYSIS PLANS

- Pilot Studies **should have:**
  - Analysis plan that directly aligns with aims
    - Descriptive
    - Confidence interval estimation
    - Hypothesis testing results: preliminary…interpret with caution; maybe increased α

# DATA ANALYSIS PLANS

- Pilot Studies **should have:**
  - Plans for how this study will inform larger study
    - What are next steps?
    - Must be very clear

# DATA ANALYSIS PLANS

- Pilot Studies **should not do:**
  - Analysis plan: “Statistical procedures as appropriate”
  - Sample size: “No sample size calculations are provided due to the pilot nature of this study”

# SAMPLE SIZE CONSIDERATIONS

- Power analyses are generally not necessary
  - In other words, you do not need to demonstrate sufficient statistical power

- Primary goal:
  - Precision of estimates to provide solid evidence to continue
    - Feasibility estimates
    - Characteristics of data/study to be used in the power analysis of the next, larger study

# SAMPLE SIZE CONSIDERATIONS

- Feasibility Measures:
  - Recruitment
  - Implementation
  - Acceptability
- Adverse Events
- Attrition

*These typically are rates – so target a sample size to obtain a sufficiently narrow 95% confidence interval around these rates*

# SAMPLE SIZE CONSIDERATIONS

What components are necessary for a power analysis?

- Sample size
- Significance level
  - Usually 0.05
- Power
  - Usually target 0.80 or 0.90
- Size of the effect of interest
  - What is clinically meaningful
- Continuous outcome: variability
- Binary outcome: rate
  - Overall or in control group
- Longitudinal data: correlations

*These should be estimated in pilots*

# EFFECT SIZES FROM PILOT STUDIES

- Power analysis for larger study should NOT be based on effect size from pilot study
  - Pilot studies are usually small → unreliable estimates of treatment effects
  - Clinically meaningful effect should be defined a priori (and should not change between pilot and larger study)

Leon, Davis, Kraemer. The role and interpretation of pilot studies in clinical research. Journal of Psychiatric Research 2011; 45: 626-629.

# REFERENCES & CONTACT INFORMATION


- Lancaster GA, Dodd S, Williamson PR. Design and analysis of pilot studies: recommendations for good practice. J Eval Clin Pract. 2004 May;10(2):307-12. doi: 10.1111/j..2002.384.doc.x. PMID: 15189396.

- Leon, Davis, Kraemer. The role and interpretation of pilot studies in clinical research. Journal of Psychiatric Research 2011; 45: 626-629.

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- Questions?  Feel free to email me at matthewgurka@ufl.edu