



PLANNING AND DESIGNING A PILOT STUDY

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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

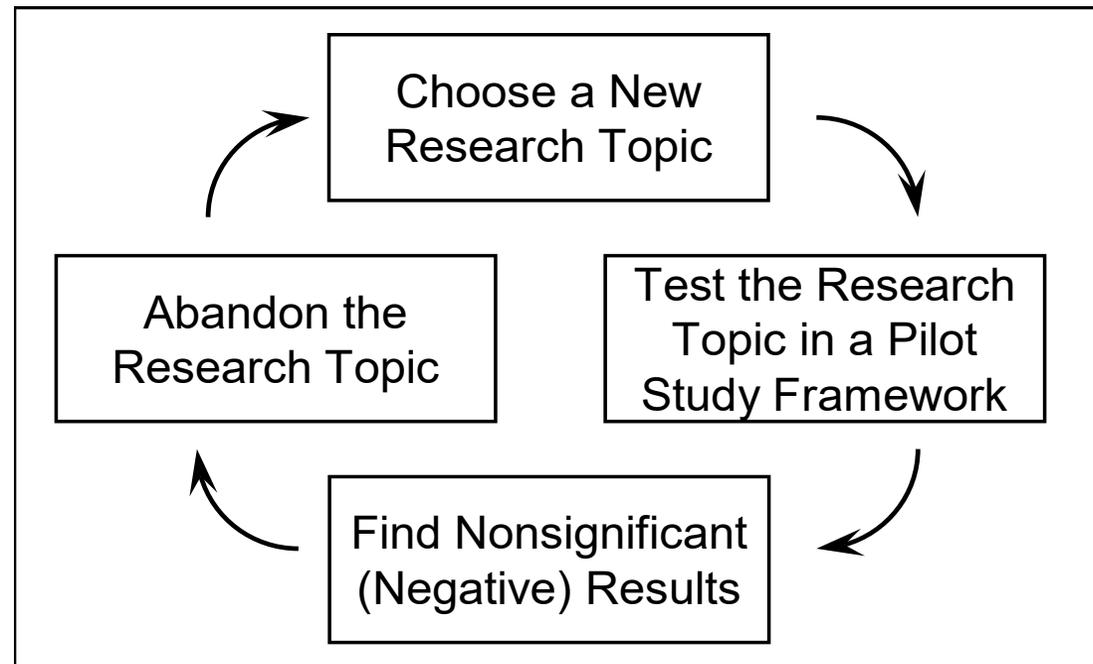
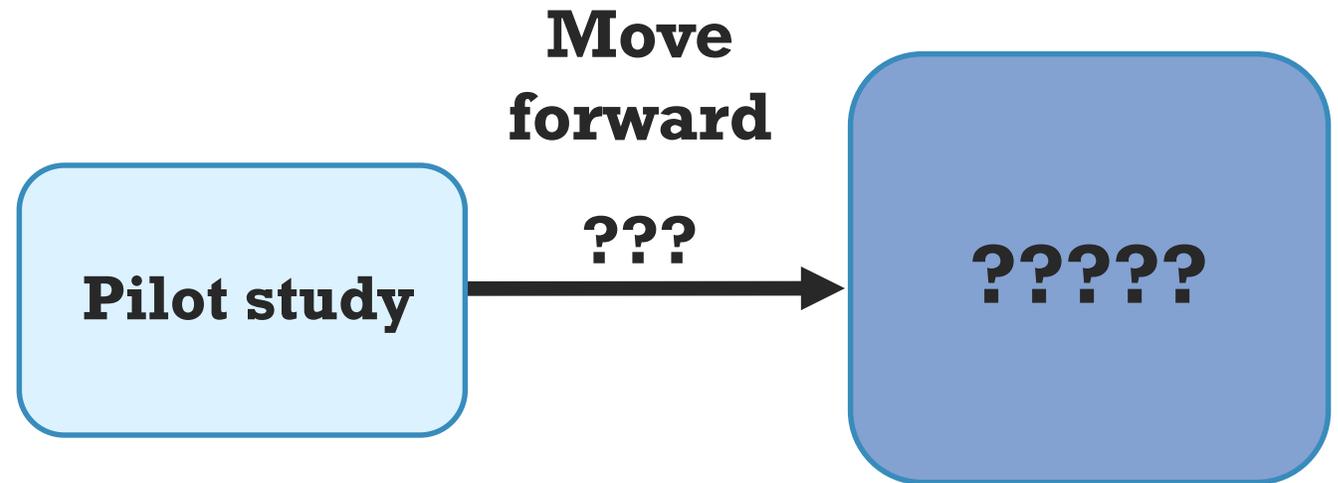


Figure from: Moore CG, Carter RE, Nietert PJ, Stewart PW. Recommendations for planning pilot studies in clinical and translational research. Clin Transl Sci. 2011 Oct;4(5):332-7. doi: 10.1111/j.1752-8062.2011.00347.x.



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



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



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.



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.



EXAMPLE PILOT

- A feasibility study of a prospective cohort following pregnant women and their children with a primary goal of measuring brain development through MRI's (starting immediately after birth)
 - With a focus on impact of substance use during pregnancy
- Aims:
 1. To assess the feasibility of recruiting patients to such a study
 2. To assess the ability to obtain successful MRI's after birth

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

