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:
WHAT OFTEN HAPPENS WITH PILOTS

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

<table>
<thead>
<tr>
<th>Category</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Screening</td>
<td>Number screened per month</td>
</tr>
<tr>
<td>Recruitment</td>
<td>Number enrolled per month</td>
</tr>
<tr>
<td>Randomization</td>
<td>Proportion screened eligible who enroll</td>
</tr>
<tr>
<td>Retention</td>
<td>Treatment-specific retention rates</td>
</tr>
</tbody>
</table>

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

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

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 $\alpha$
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

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

• Adverse Events

• Attrition
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)

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
EXAMPLE PILOT: SAMPLE SIZE JUSTIFICATION

• Approach n=25 eligible subjects

• If n=20 of 25 (i.e. 80%) of eligible patients who are approached agreed to participate
  • 80% enrollment rate (with a 95% CI ranging from 59% to 93%)
  • Evidence that at least a majority of patients would be willing to participate
EXAMPLE PILOT: SAMPLE SIZE JUSTIFICATION

• Enroll 10 women who are using substances during pregnancy

• If we obtain successful MRI’s on 8 of the 10 subjects:
  • 80% success rate (95% CI: 44% to 97%)
  • Some evidence that obtaining MRI’s is feasible in this patient population?
REFERENCES & CONTACT INFORMATION


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