Title

Research Methodology Tip - Objectives of Feasibility and Pilot Studies: Asking the Right Question

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Organisation

Tan Tock Seng Hospital

Healthcare Family Group

Healthcare Administration

Applicable Specialty or Discipline

Clinical Research

Content Category

Training & Education

Learning Theories, Methodologies & Framework

Keywords

Feasibility Study, Pilot Study

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[Extracted from CRIO Newsletter (Issue 03) Dec 2021]

Research Methodology Tip

Objectives of Feasibility and Pilot Studies: Asking the Right Question

What is the purpose of such studies?

Within the scope of feasibility and pilot studies, any uncertainties around the key elements of a large scale conclusive main trial testing a novel intervention can be studied and resolved, if possible. The key elements include the intervention strategy, intended population, study design, study setting, delivery of the intervention and assessments of outcomes of interest.

The results of such studies, when used appropriately, enhance the success prospect of the main trial and are often a pre-requisite in grant applications for studies asking for funding. In figure 1, we present a summary of the specific endpoints that can be measured and potentially be used as benchmark for success of such studies[1,2].

Other than the process, resource and management endpoints indicated here, pilot studies may estimate some relevant scientific endpoints, such as safety, tolerability and effectiveness/efficacy of the tested intervention, as well. However, any conclusions regarding such endpoints must be made with caution when it comes to the pilot studies; reasons are discussed later.

What are some of the typical designs that can be used for such studies?

Feasibility and pilot studies may implement any of the qualitative (such as individual interviews, focus groups etc) or quantitative (such as surveys collecting numerical data, single arm, non-randomized and randomized multiple arm etc) research methods, or even a mixed-method design and it should be driven by the specific aims, planned relevant endpoints of the study and available resources. We, the clinical research support team, help the investigators refine their study aims and choose an optimal design appropriate to answer the specific research questions. It's possible to secure seed-funds for feasibility and pilot studies. Well-conceived, and properly executed feasibility and pilot studies are very much likely to get published as original research article, regardless of the study outcome, in peer-reviewed journals.

Figure 1: Specific outcomes and endpoints of feasibility and pilot studies

Process This assesses the feasibility of the processes that are key to the success of the main study	**Resources** This deals with assessing time and resource problems that can occur during the main study	**Management** This covers potential human and data management problems
• Monthly rates of recruitment, cumulative refusal and retention rates, cumulative treatment fidelity and adherence rates. • Blinded assessment procedures.	• Length of time to fill out all the study forms, average time delay from screening to enrollment, other process times.	• What challenges do study personnel have? • Any management issues? • Monitoring and regulatory reporting procedures.
• Eligibility criteria (Is it obvious who meets and who does not meet the eligibility requirements? Are the eligibility criteria sufficient or too restrictive?)	• Determining capacity of the setting: hardware/software, equipment readily available, when and where it is needed? • Troubleshooting techniques available?	• Is there enough room on the data collection form for all of the data you expect to receive? • Are there any problems entering data into the computer?
• Development and/or validation of study questionnaires or data collection tools • Do subjects provide multiple answers, qualified answers, or unanticipated answers to study questions, or leave the space blank?	• Determining centre willingness and capacity (commitment levels, centre-specific capacity issues). • Can I randomize my target population? • Will the study participants overload the waiting room?	• Were any important data values forgotten about? • Matching of data coming from different sources. • Do data show too much or too little variability?

How about sample size? Is there any rule of thumb, such as n = 12 or 30, for these studies?

Any study plan needs to have a proper justification of the number of subjects it's aiming to recruit. Feasibility and pilot studies are no exception. Sample size justification is study-specific and should be based on the defined feasibility endpoints, benchmark for the success of the study, and the availability of required resources (i.e., recruitment potential, time, manpower, budget etc). In some cases, statisticians consider either a precision based or a standardized effect size-based calculation in the justification too[3]. We don't believe in any rule of thumb for this matter and always try to work out a scientific and pragmatic justification in consultation with the study team after reviewing the draft protocol.

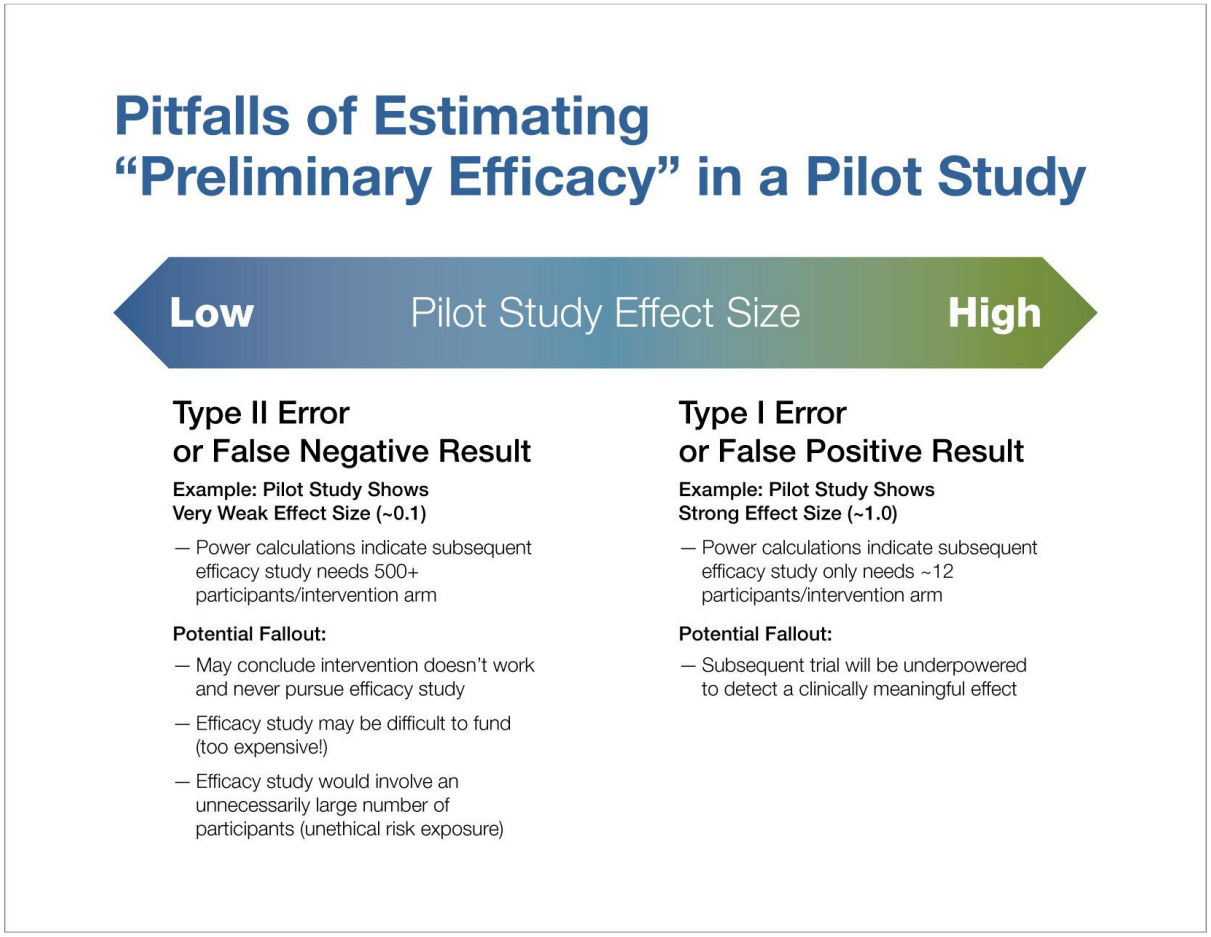
What are the common misconceptions around feasibility and pilot studies?

First of all, a study testing hypothesis on effectiveness/efficacy and safety of an intervention but underpowered due to the resource constrains should never be labelled as pilot study. Study protocol must communicate how the results of this study will inform the independent main trial.

Secondly, the small sample size of pilot studies, due to great amount of uncertainty, may not be adequate to make any sound conclusions on the efficacy of the intervention. Similarly, estimates of endpoints such as safety and tolerability found in pilot studies may be unstable as well. It can only give some rough preliminary idea on extremely common and severe safety concerns, but the intervention can't be declared safe using such small data, unless tested in bigger scale study.

Finally, too much reliance on the unstable estimate of the effect size found in the pilot study in order to calculate the sample size for the main trial can be potentially dangerous. It may lead to either an underpowered or unnecessarily expensive main trial. Rather, the upper limit of the confidence interval of the variability (i.e., standard deviation) around the effect

size estimated in pilot study, along with a clinically meaningful effect, should be used to size the main trial[4].



Source: National Centre for Complementary and Integrative Health, National Institute of Health, USA

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