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A Study on Scientific Writing: The Pilot Study

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A concern in all research universities is the lack of attention and information about pilot studies in its textbooks. This brief discussion about the definition of a pilot study may serve as a resource for investigators and authors who are designing pilot studies and reporting data from pilot studies.

Specifically, as a result of the new DAOM programs that have become part of acupuncture and Oriental medicine (AOM) academic institutions, many new AOM researchers and investigators are writing and publishing in the field. AOM school clinics have become a place for these new investigators to get their feet wet and do clinical research by designing and conducting small pilot studies.

We therefore need to ask: **What is the purpose of a pilot study?** In their manuscript *The Role and Interpretation of Pilot Studies in Clinical Research*, Leon et al. state that “Prior to initiating a full scale RCT an investigator may choose to conduct a pilot study in order to evaluate the feasibility of recruitment, randomization, retention, assessment procedures, new methods, and/or implementation of the novel intervention.”

The pilot study is a very important part of the research process. It is done as a first step towards conducting a larger study. If the pilot study flows well, and the research team works well together, then both the researchers and the grantors know that the study team can successfully conduct the study again on a much larger scale.

Reasons for Conducting Pilot Studies

(From Thabane et. al, A Tutorial on Pilot Studies (Table 2)):

Assess the feasibility of the processes that are key to the success of the main study:

Recruitment rates • Retention rates • Refusal rates • Failure/success rates • (Non-) compliance or adherence rates • Eligibility criteria: Is it obvious who meets and who does not meet the eligibility requirements? Are the eligibility criteria sufficient or too restrictive?

Understanding of study questionnaires or data collection tools:

Do subjects provide no answer, multiple answers, qualified answers, or unanticipated answers to study questions?

Resources:

Assessing time and resource problems that can occur during the main study • Length of time to fill out all the study forms

Determining capacity:

Will the study participants overload your phone lines or overflow your waiting room? • Determining process time: How much time does it take to mail out and receive response from a thousand surveys? • Is the equipment readily available when and where it is needed? • What happens when it breaks down or gets stolen? • Can the software used for capturing data read and understand the data? • Determining center willingness and capacity: Do the centers do what they committed to doing? Do investigators have the time to perform the tasks they committed to doing? Are there any capacity issues at each participating center?

Management: (This covers potential human and data management problems.)

What are the challenges that participating centers have with managing the study? • What challenges do study personnel have? • Is there enough room on the data collection form for all of the data you receive? • Are there any problems entering data into the computer? • Can data coming from different sources be matched? • Were any important data values forgotten about? • Do data show too much or too little variability?

Scientific:

This deals with the assessment of treatment safety, dose, response, effect and variance of the effect • Is it safe to use the study drug/intervention? • What is the safe dose level? • Do patients respond to the drug? • What is the estimate of the treatment effect? • What is the estimate of the variance of the treatment effect?

What a Pilot Study Cannot Do

A pilot study is *not* conducted for testing a hypothesis.

A pilot study *cannot* assess the effectiveness of an intervention or new treatment! There are simply not enough subjects/patients and data for statisticians to calculate that the treatment was efficacious.

The pilot study *cannot* determine safety of an intervention.

Considerations in Pilot Study Design

When we design a research study, we must focus on aims and objectives. If our institution requires a letter of intent, then the aims and objectives need to be clearly stated.

Aims: What questions we wish to answer. Why are we doing the study?

Objectives: How we intend to gather data to answer the question. Questionnaires? Bloodwork?

A pilot study should have at least two aims.

Aim 1: Is there clinical benefit to using [] for the treatment of []?

Aim 2: To determine the feasibility of recruiting [] patients for this trial.

Primary Questions (Aims) that can be Answered in a Pilot Study

(These would be stated in the letter of intent, protocol and when the data is reported in a manuscript.)

Can the target subjects be recruited for this study?

Treatment fidelity: Will the subject comply with the study protocol? Will they come to all their acupuncture treatments? Will they take their herbs? Will they complete all the questionnaires? Will the treatment be administered properly?

Personal Questions for the Research Team and Grantors:

(These concerns would not go into the letter of intent or protocol but may be discussed as useful information when reporting data in both manuscripts and oral presentations.)

Can I assemble a team that can work well together?

Accounting: If seed money is used, will the grant accounting/purchasing process work?

Statistics: Will the stat plan, database, data collection, data entry systems work?

What can we learn from this study that we can modify/simplify/improve when we conduct the study on a larger scale?

What Type of Study is a Pilot Study?

A pilot study may fit into the category of a Phase I or Phase II clinical trial. Data from the pilot study may be used to conduct a larger Phase II or Phase III study.

The NIH.gov website explains the different phases in research:

<http://www.nlm.nih.gov/services/ctphases.html>

Clinical trials are conducted in a series of steps, called phases, and each phase is designed to answer a separate research question.

- **Phase I:** Researchers test a new drug or treatment for the first time in a small group of people to evaluate its safety, determine a safe dosage range, and identify side effects.
- **Phase II:** The drug or treatment is given to a larger group of people to see if it is effective and to further evaluate its safety.
- **Phase III:** The drug or treatment is given to large groups of people to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the drug or treatment to be used safely.
- **Phase IV:** Studies are done after the drug or treatment has been marketed to gather information on the drug's effect in various populations as well as any side effects associated with long-term use.

Reporting Data from a Pilot Study

When reporting the data from a pilot study, it is important to view and discuss the trial as a feasibility study. It's important to report positive findings, but we must in no way claim that the findings are evidence for the use of the new treatment. If the results are positive, it is important to report that the treatment shows promise, and further studies should be done to confirm effectiveness.

When reporting the data from an acupuncture or herb study, it is customary to state that no adverse events have occurred (if no events occurred), but authors should be careful not to claim that the treatment or intervention is safe because there is not enough data to support claims of safety in the specific target population using the intervention in the pilot study. It is sufficient to report simply that "no adverse events were reported."

Always discuss limitations learned from or about the pilot study. The limitations are due to the small sample size. There is not enough data to claim effectiveness or safety of the intervention. We may report that the intervention showed promise, and it might be considered as a treatment option, and no adverse events were reported. But we *cannot* report that the intervention was successful, effective, or safe.

References and Recommended Reading

Leon A, Davis L, Kraemer H. The role and interpretation of pilot studies in clinical research. *J Psychiatr Res*. May 2011;45(5):626–629. <http://www.ncbi.nlm.nih.gov/pmc/articles/PMC3081994/>

Thabane L, Ma J, Chu R, et al. A tutorial on pilot studies: The what, why and how. *BMC Medical Research Methodology* 2010;10:1 <http://www.biomedcentral.com/1471-2288/10/1>