

## **Pilot Study Checklist**

- At least one of the following reasons to conduct the study applies
  - Study administration
  - Data management
  - Scientific
  
- Aims and objectives are clearly stated
  
- Collected data are consistent with goals
  
- No statistical hypothesis is tested
  
- Sample size is justified (not necessarily in a statistical sense)
  
- The way in which the data collected will be used in the design of a larger study has been addressed
  
- This study will answer the question of whether a full scale trial/experiment is worth pursuing
  
- Criteria that will determine continuation to a larger study are specified

## **Other recommendations and thoughts**

Distinguish between

- Toy problems for learning experience
- Identifying feasibility answers
- Getting “preliminary data” (why?)

Always identify

- What the goal of the study is
- How it will be determined whether the goal is met
- What steps need to be taken to meet the goal

What is innovative or novel.

A poorly designed pilot study can be used as an example to train investigators in designing experiments.

## **Example of a badly designed pilot study**

*Prior to a larger clinical trial, a pilot study involving 10 patients is proposed. The goal of the project is to investigate how well patients will tolerate wearing a new ambulatory heart monitor while receiving an experimental medication. Data will be downloaded from the monitors and will be analyzed using a t-test procedure for comparison of post-treatment heart rate to pre-treatment heart rate.*

- The main goal was to assess tolerability but there is no measure defined for it. No data to address the main goal is collected.
- There is an implicit hypothesis tested.
- Sample size is not justified and may be driven by the desire to detect a difference.

If the goal is to estimate tolerability, where tolerability is defined as a dichotomous variable. Then the investigator needs to describe a criterion for unacceptable tolerability. The closer the intolerability is to zero, the larger the sample size needs to be.

Suggest a two tiered review. Statistician need only review protocols that have scientific merit.

## **References**

<http://www.ctspedia.org/do/view/CTSpedia/SuccessStory041>

Moore CG, Carter R, Nietert PJ, Stewart PW (2011). Recommendations for planning pilot studies in clinical and translational research. *Clinical and Translational Science*, 4(5): 332-337.