



# ***UAB Center for Clinical and Translational Science***

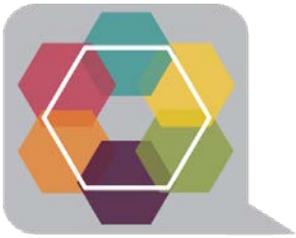
Director, Robert P. Kimberly, MD

## **Design and Analysis of Pilot Studies**

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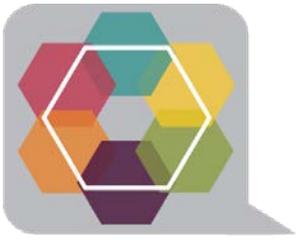
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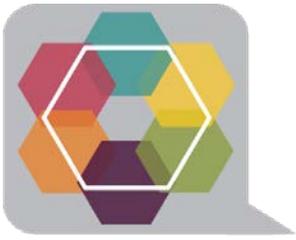
## What is a Pilot Study?

- Borrowing from Thabane et al (2010), a pilot study can be defined as a ‘small study often done to assist the preparation of a larger, more comprehensive study.’
- The Free Dictionary defines a Pilot Study as a small-scale experiment or set of observations undertaken to decide how and whether to launch a full-scale project.



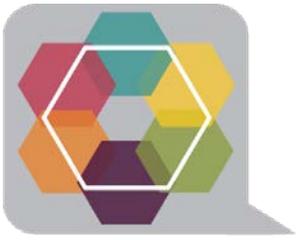
## What is a Pilot Study?

- Almost all definitions found on the Internet will focus on the fact that a pilot study is a small study that preceded a larger study.



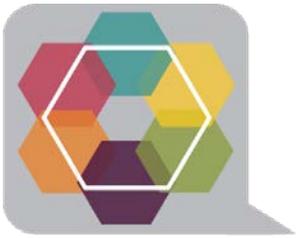
## What is a Pilot Study?

- It is very very important to note that the goals of Pilot Study are to demonstrate the feasibility of key components (recruitment, consent, randomization, data collection) and to estimate key parameters (standard deviation) need for a larger trial.



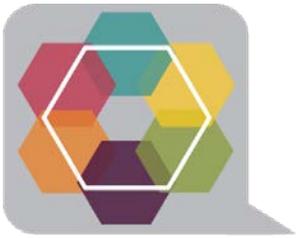
## What is NOT a Pilot Study?

- A Pilot Study is NOT simply a repackaged version of a larger study but with a reduced sample size you can afford.
- A Pilot Study is NOT simply a scientific code/statistical jargon for ‘A study we wish to do but want to avoid criticisms over statistical power issues.’



## Excellent References

- Thabane et al (2010). A tutorial on pilot studies: the what, why, and how. BMC Medical Research Methodology 2010 10:1.
- Lancaster et al (2004). Design and Analysis of Pilot Studies: recommendations for Good Practice. Journal of Evaluation in Clinical Practice, 10, 2, 307-312.



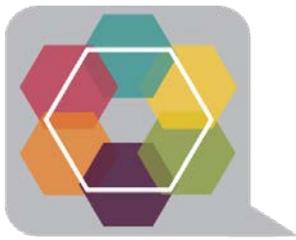
## Excellent References

- Leon et al (2011). The Role and Interpretation of Pilot Studies in Clinical Research. *Journal of Psychiatric Research* May 45(5): 626-629.



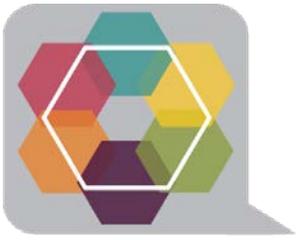
# Reasons to Conduct Pilot Trials

Reasonable Aims for a Pilot Study	Reasonable Aims for R01/Larger Study
To Demonstrate Feasibility of Collecting Diary Data	To Test for Outcome Differences of Two Treatments based upon Diary Data
To Demonstrate Ability to Recruit Elderly Subjects to Intensive Weight Training	To Test for Differences in Bone Mineral Density between Individuals randomized to aerobic vs Intensive weight training.
To Estimate Completion Rate of Elderly Subjects conducting Intensive Weight Training	To Test for Differences in Bone Mineral Density between Individuals randomized to aerobic vs Intensive weight training.
To Estimate Adherence in a given population to medication	To Test for Differences in adherence rates between two motivational strategies.



## Reasons to Conduct Pilot Trials

- Do you see the difference. The Pilot Study is a study to demonstrate the feasibility of key components of a larger future trial.
- The Pilot Study is a study that can allow collection of data to guide the design of a larger future study.
- However, hypothesis testing and inferential procedures are most appropriate for the larger study.



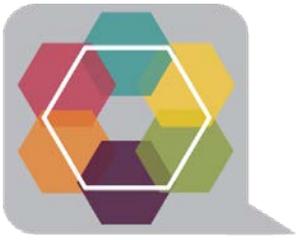
## Reasons to Conduct Pilot Trials

- Just like a large research project, a pilot study must have well defined goals/aims. However, these aims should be focused upon feasibility or estimation.
- The simple way to identify the aims of the pilot is to ask ‘What must be demonstrated/refined to make the larger trial more likely to succeed?’



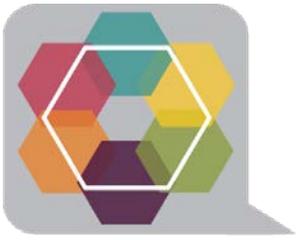
## Design of Pilot Studies

- So well articulate aims are essential for a pilot study as well as a ‘vision for future studies.’
- However, the lack of hypothesis testing and inferential procedures leads to a frustrating issue. The primary method of sample size determination, power, is not helpful.



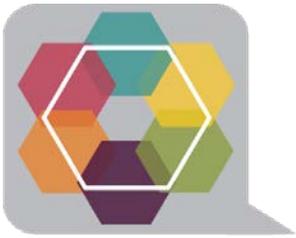
# Design of Pilot Studies

- The question becomes how should one decide about sample size for the pilot trial.
- Often sample sizes are justified by using a confidence interval approach where the sample size is chosen to provide an acceptable level of precision.



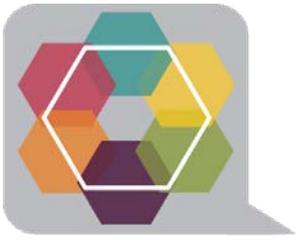
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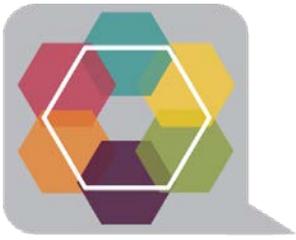
## Example of Goals

- If 27 or more of the 30 subjects complete 85% of the intensive weight lifting sessions, the intervention will be considered feasible.



## Example of Goals

- It is very important to set benchmarks that must be met within the pilot studies.
- When these benchmarks are met, then one considers that the instruments and methods are adequate to implement in a future trial.
- Where do these benchmarks come from? From discussion among peers, clinical opinion.



## Example of Goals

- Where do these benchmarks come from? From discussion among peers, clinical opinion.



## Common Questions for Pilot Studies

Question: May I include my pilot data into my larger study?

Answer: Maybe! If appropriate statistical methods are used (Internal Pilots) and methods are constant, it is possible. Talk to a statistician.



## Common Questions for Pilot Studies

Question: Can I randomize within a pilot study?

Answer: Absolutely! Both the demonstration of ability to consent and willingness to accept random assignment could be crucial for the review of a larger study.



## Common Questions for Pilot Studies

Question: If I randomize, can I use results of my pilot trial to estimate a treatment difference and use that in my power calculation?

Answer: NO! You may use the variance estimates but the minimum clinically relevant difference should be based on clinical opinion.



## BERD Consultations

- Remember the CCTS Biostatistics, Epidemiology, Research Design (BERD) methodologists offer 2 walk-in clinics per week.
- First weekly clinic is every Monday 10-2, Edge of Chaos.
- Second weekly clinic is every Wednesday 11:30 - 1, PCAMS.



## BERD Consultations

- Walk-in clinics are ideal for simple analysis advice for paper submissions, basic data management issues, and power calculations.
- The BERD provides one-on-one consultations for R-mechanisms or K mechanisms that you are planning to submit.



## How to contact the BERD

- Email the Research Commons: [ccts@uab.edu](mailto:ccts@uab.edu)  
[www.uab.edu/ccts](http://www.uab.edu/ccts)