

PILOT STUDIES

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Trial Development Models

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WHAT IS A PILOT STUDY?



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A smaller version of a future randomized trial



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A preliminary test of efficacy



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A study to assess the feasibility of a protocol



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A study to estimate an effect size



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WHAT A PILOT STUDY IS NOT A Wake-Up Call

ibtn
international
behavioural
trials network

PERSPECTIVES

Caution Regarding the Use of Pilot Studies to Guide Power Calculations for Study Proposals

Helena Chmura Kraemer, PhD; Jim Mintz, PhD; Art Noda, MS; Jared Tinklenberg, MD; Jerome A. Yesavage, MD

Arch Gen Psychiat 2006;63:484-9



WHAT A PILOT STUDY IS NOT

The Wake-Up Call

Pilot studies produce unstable estimates of efficacy.

They are not the basis for determining:

- The sample size of a trial;
- If a trial should be conducted.

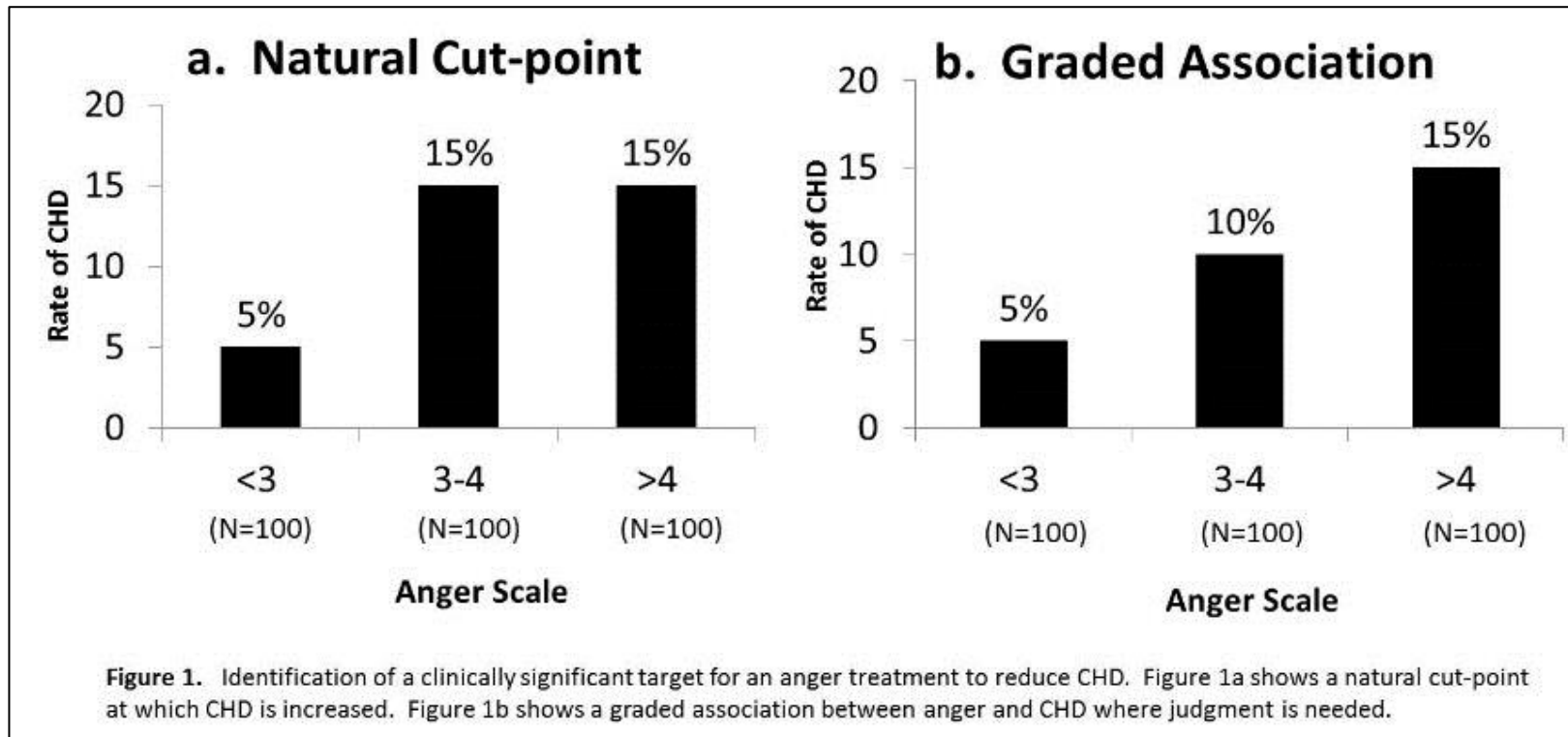
*“Studies worth performing are aborted.
Studies not aborted are underpowered.”*

Kraemer et al., 2006



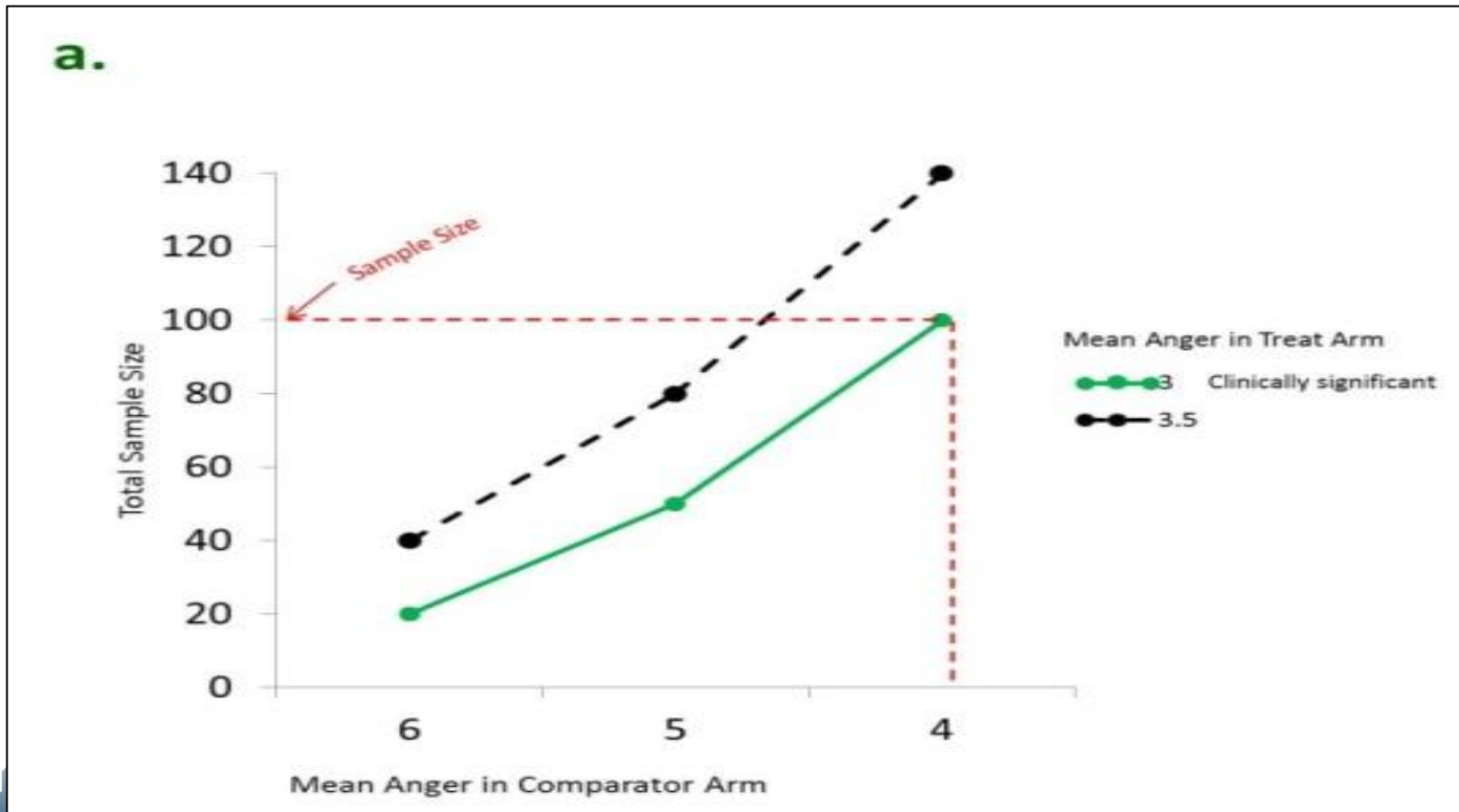
HOW DO YOU DETERMINE SAMPLE SIZE?

Find Clinically Significant Target



HOW DO YOU DETERMINE SAMPLE SIZE?

Preserve Clinical Significance



HOW DO YOU DETERMINE WHETHER A TRIAL SHOULD BE CONDUCTED?

Significance

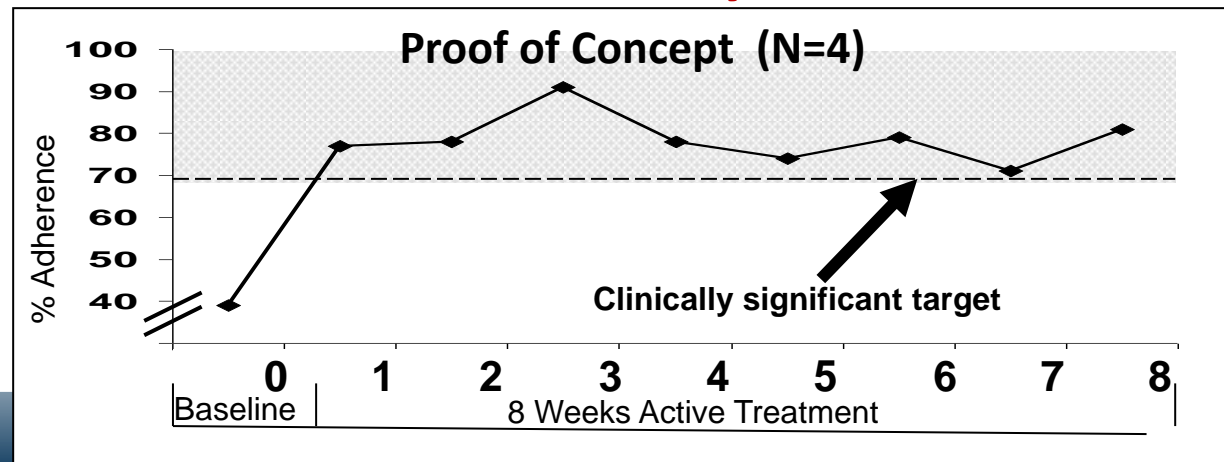
Table 3. Significant clinical problem	
Disease:	<ul style="list-style-type: none"> • Affects many • Affects a subgroup • Is increasing • Is increasing in a subgroup • Is not decreasing • Is costly
Current Treatment:	<ul style="list-style-type: none"> • No current treatment • Not very effective • Uncertain effectiveness (clinical equipoise) • Effective but adherence is poor • Effective but side effects are serious
New Treatment:	<ul style="list-style-type: none"> • Shows promise in preliminary work • Targets novel risk factor • Targets novel pathway • Less costly than standard • Fewer side effects

Credibility



- Grounded in science of behavior change.
- Systematically developed and refined

Plausibility



WHAT IS A PILOT STUDY?

CONSORT Guidelines

Eldridge *et al.* *Pilot and Feasibility Studies* (2016) 2:64
DOI 10.1186/s40814-016-0105-8

Pilot and Feasibility Studies

RESEARCH

Open Access

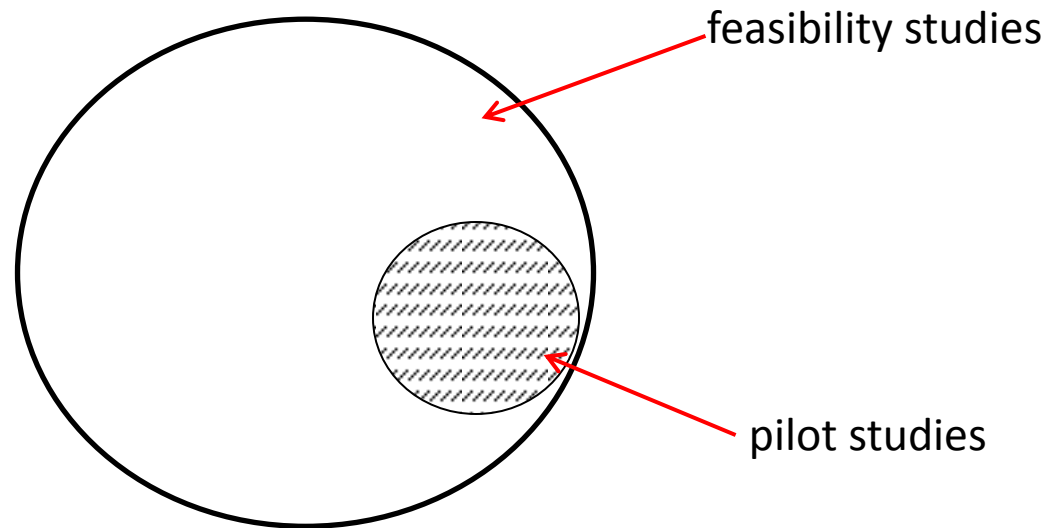
CONSORT 2010 statement: extension to randomised pilot and feasibility trials



Sandra M. Eldridge^{1*}, Claire L. Chan¹, Michael J. Campbell², Christine M. Bond³, Sally Hopewell⁴, Lehana Thabane⁵, Gillian A. Lancaster⁶ and on behalf of the PAFS consensus group

PILOT VS. FEASIBILITY PILOTS

CONSORT Guidelines



WHAT IS A PILOT STUDY?

CONSORT Guidelines

Predecessors to a future clinical trial

Aim is feasibility. Whether a future trial:

Can be done;

Should be done; and, if so,

How.

A smaller scale of the future clinical trial.



IMPORTANCE OF A PILOT STUDY

Studies Implementation

TREATED

CONTROLS

GOAL

**Attendance at dance
class \geq 3 days/week**

***Read all mailed
materials***

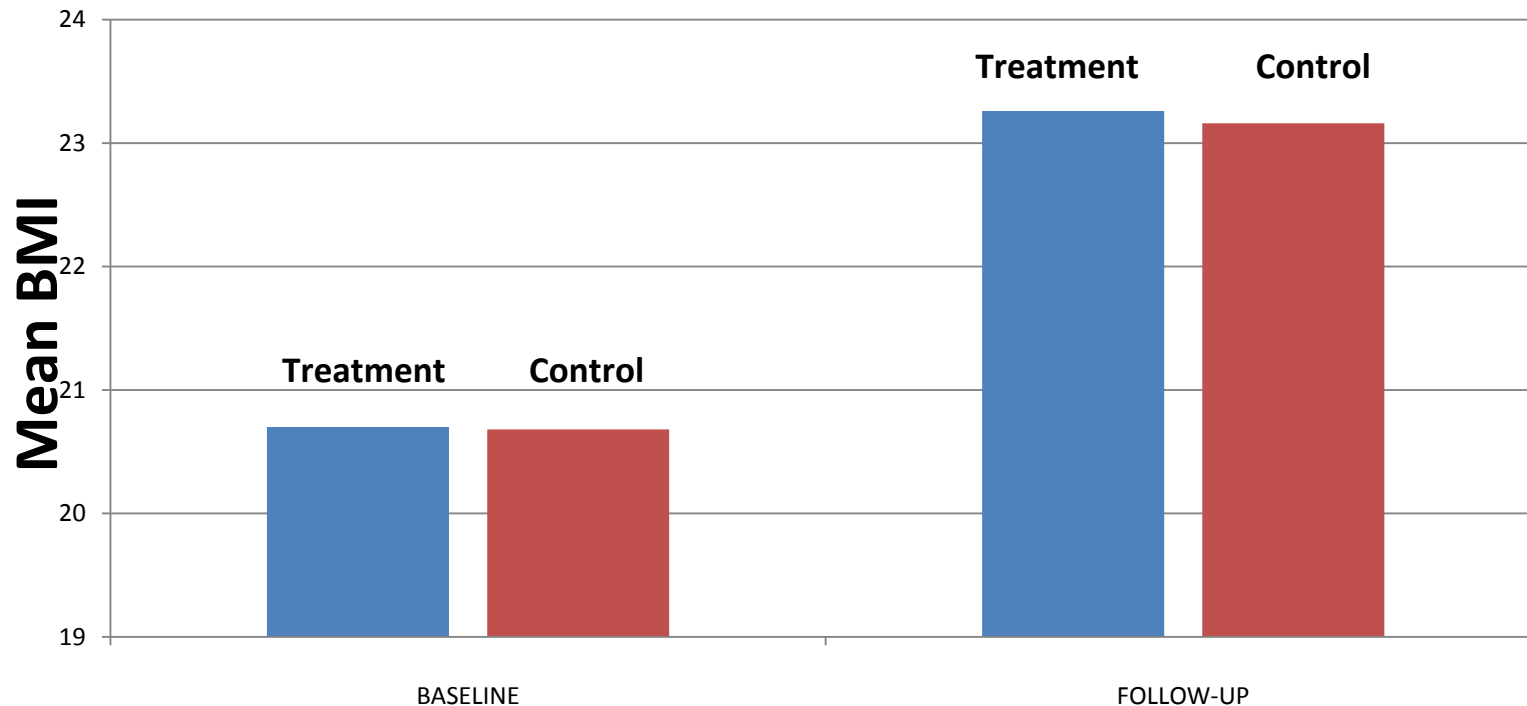
ACHIEVED

33%

66%

IMPORTANCE OF A PILOT STUDY

Inability to Implement Results in a Null Trial



PRODUCT OF FEASIBILITY PILOT STUDY

Source	Accessible/ Week	Eligible (55%)	Willing (70%)	Passed Screen (90%)
Recruitment Site #1	9	5.0	3.5	3.2
Recruitment Site #2	7	3.9	2.7	2.4
TOTALS	16	8.9	6.2	5.6

Expected Rate: 5.6/week

Number Subjects Needed: 30

Number Weeks Needed: $30/5.6 = 5.4$ weeks

(double the estimate)

Time Needed for Recruitment: 5.4 Weeks x 2 = 12 Weeks



WHO CARES ABOUT A PILOT STUDY?

Investigators	To implement a trial they designed
Reviewers	Reassurance that the trial is feasible
The Research Community	Only if the results offer generalizable lessons.



SUMMARY

Science is progressive.

Pilot studies are integral to progression

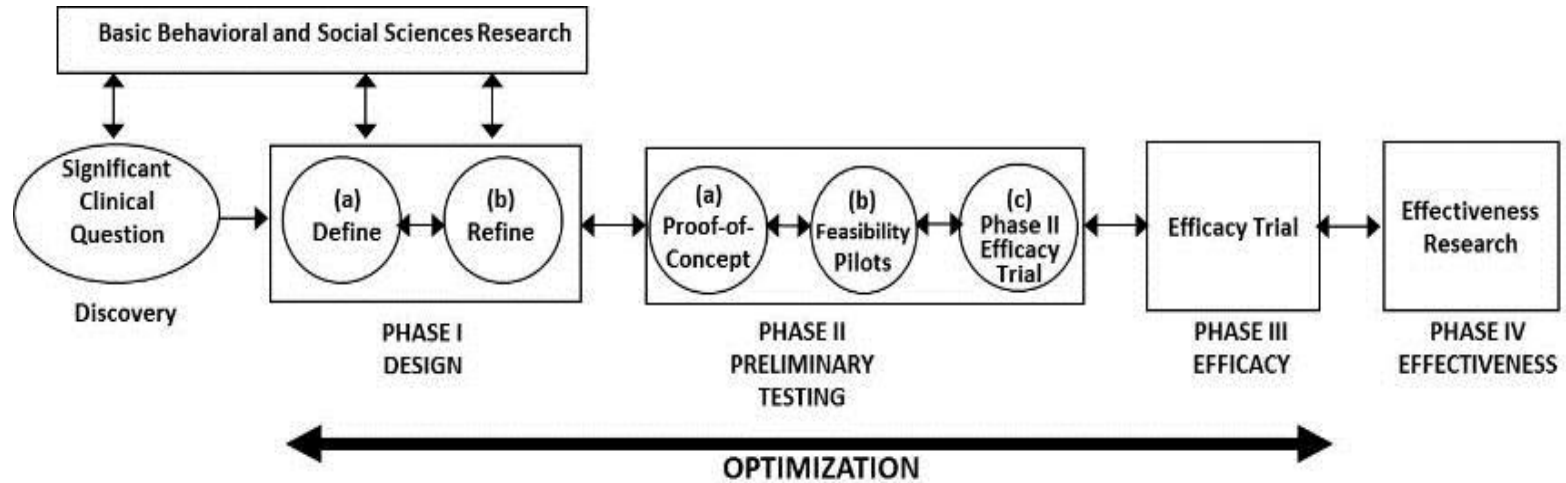


Figure 1. The ORBIT Model for Behavioral Treatment Development

Czajkowski, Powell et al., 2015;
Powell, Freedland & Kaufmann
(in press)