PILOT STUDIES

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Trial Development Models

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والمحمد والمحاج المتركب والمتناقين والمتعارية أتراجع والمتركب أتراجع ومخالفاته المتعارية والأكاري

المحمد ومحمد المتحاد فالمحاصين والأمريس وبالمحاصل الالمحمد ومرتبع والمحاد المربوب

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المتحد ومتحا فتنافأ فالمانيج والشريقين ووالواليا المتقاصين والمروا والمتعاط



A smaller version of a future randomized trial



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A smaller version of a future randomized trial A preliminary test of efficacy





- A smaller version of a future randomized trial
- A preliminary test of efficacy
- A study to assess the feasibility of a protocol





- A smaller version of a future randomized trial
- A preliminary test of efficacy
- A study to assess the feasibility of a protocol
- A study to estimate an effect size





A smaller version of a future randomized trial

A preliminary test of efficacy

A study to assess the feasibility of a protocol

A study to estimate an effect size

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WHAT A PILOT STUDY IS NOT A Wake-Up Call



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 <u>NOT</u> 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

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HOW DO YOU DETERMINE SAMPLE SIZE? Find Clinically Significant Target



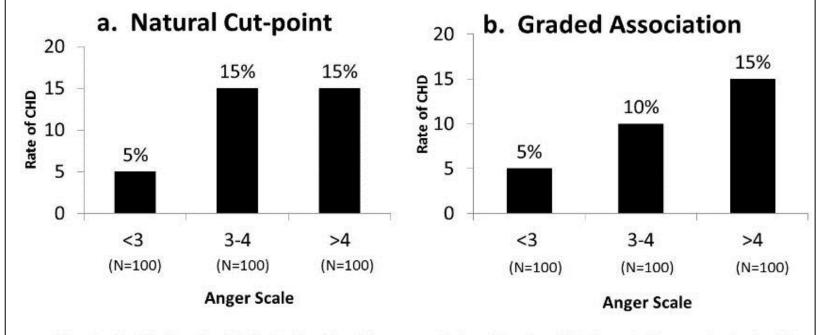
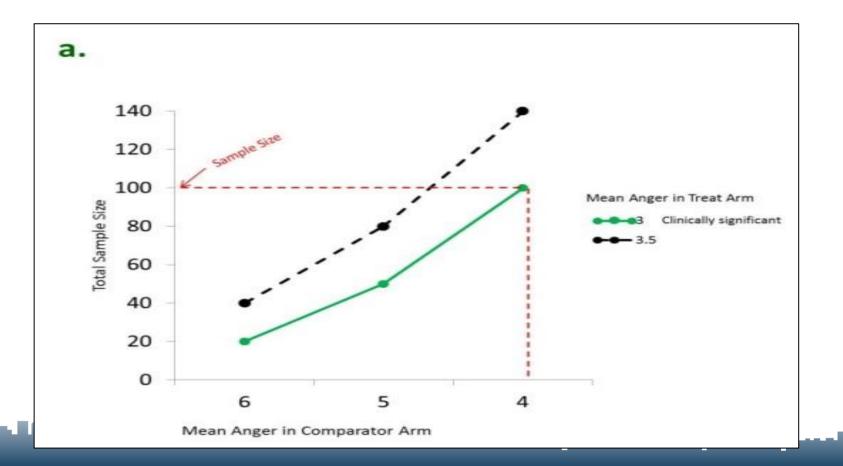


Figure 1. Identification of a clinically significant target for an anger treatment to reduce CHD. Figure 1a shows a natural cut-point at which CHD is increased. Figure 1b shows a graded association between anger and CHD where judgment is needed.



HOW DO YOU DETERMINE SAMPLE SIZE? Preserve Clinical Significance

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ومستحدثا المستحدي والمراجعين والمرافعية لبالا استخلاصها والمراجعاتيان والا

HOW DO YOU DETERMINE WHETHER A TRIAL SHOULD BE CONDUCTED?

Significance

Disease:	Affects many
	 Affects a subgroup
	 Is increasing
	 Is increasing in a subgroup
	 Is not decreasing
	• Is costly
Current Treatment:	 No current treatment
	 Not very effective
	 Uncertain effectiveness (clinical equipoise)
	 Effective but adherence is poor
	 Effective but side effects are serious
New Treatment:	Shows promise in preliminary work
	 Targets novel risk factor
	 Targets novel pathway
	 Less costly than standard
	 Fewer side effects

Credibility



--Grounded in science

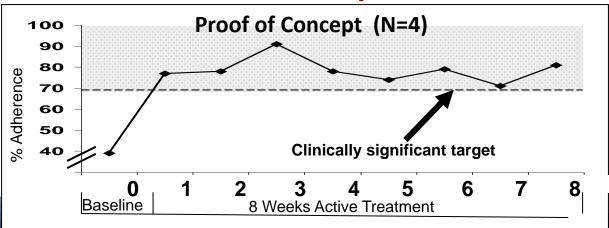
- -Grounded in science of behavior change.
- --Systematically developed and refined

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Plausibility



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WHAT IS A PILOT STUDY? CONSORT Guidelines

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

Pilot and Feasibility Studies

RESEARCH

Open Access



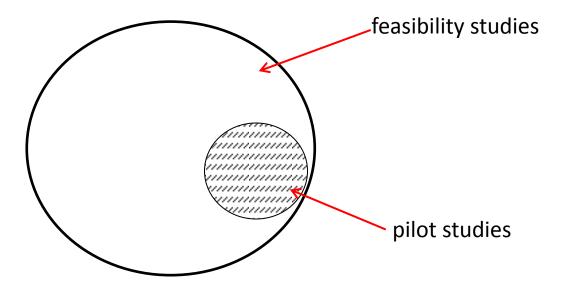
CONSORT 2010 statement: extension to randomised pilot and feasibility trials

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

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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.

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IMPORTANCE OF A PILOT STUDY Studies Implementation



TREATED

CONTROLS

GOAL Attendance at dance class <u>></u> 3 days/week Read all mailed materials

ACHIEVED 33% 66%

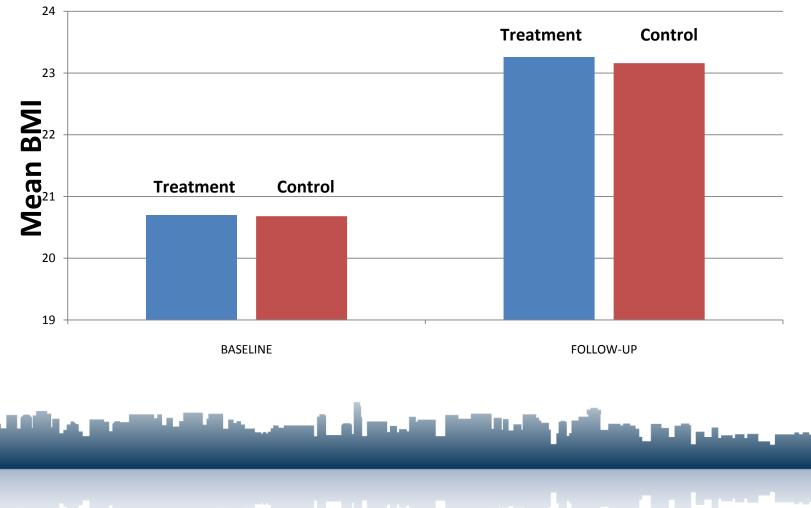
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والمحاجب والارتباط والمتحاج والمحاج والمحاج والمحاج والمحاج والمحاج والمحاج والمحاج والمحاج والمحاج والمحاج

Robinson, Stanford GEMS

IMPORTANCE OF A PILOT STUDY Inability to Implement Results in a Null Trial

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PRODUCT OF FEASIBILITY PILOT STUDY

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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: Number Subjects Need Number Weeks Needed		5.4 weeks	(double the e	stimate)			
Time Needed for Recruitment: 5.4 Weeks $x \dot{2} = 12$ Weeks							
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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

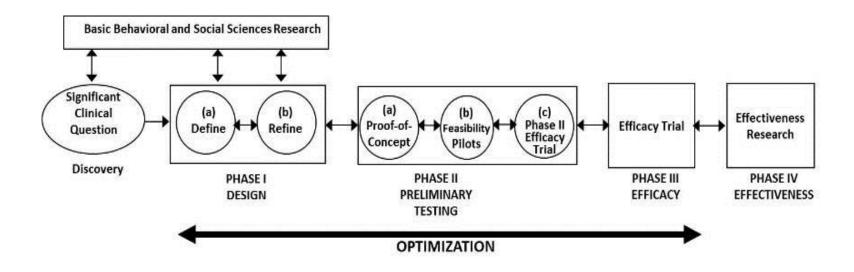


Figure 1. The ORBIT Model for Behavioral Treatment Development

والمتناقي وتمرو أمرار

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

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