

May 16-18  
Montreal, Canada

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# CONFERENCE

10th Anniversary Edition

# BEHAVIORAL CLINICAL TRIALS: PAST, PRESENT, AND FUTURE

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# Summer Institute on Randomized Behavioral Clinical Trials

## U.S. National Institutes of Health

### Class of 2004

## FELLOWS

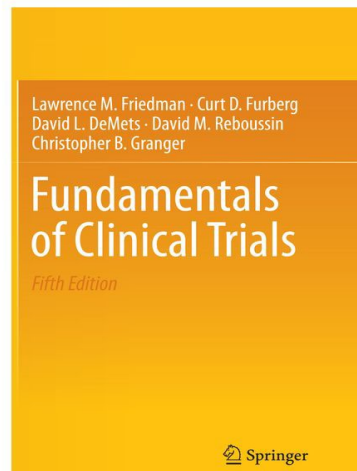


## FACULTY



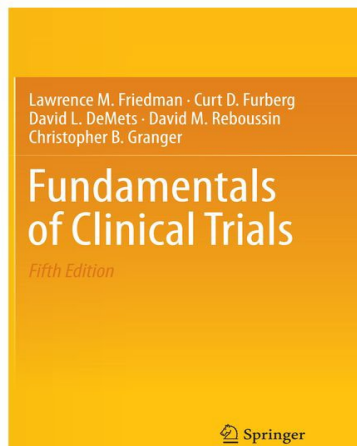
## “Rules” for Randomized Drug Trials (from Epidemiology)

- Design: Double-blind
- Eligibility: Adherence, Safety
- Sample size: Conservative
- Outcome: Single primary outcome
- Analyses: Intent-to-Treat
- *Phase III* efficacy trial required



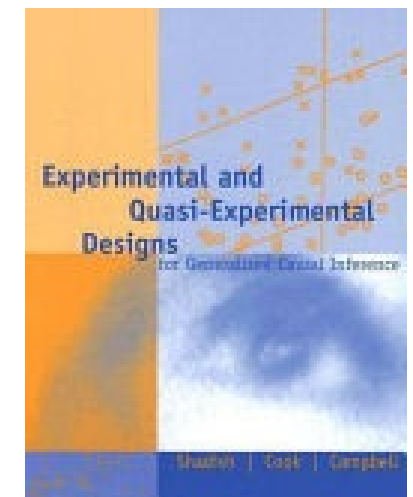
## “Rules” for Randomized Drug Trials (from Epidemiology)

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## State of the Art in Randomized Behavioral Trials (from Experimental Psychology)

- Design: Unblinded, Single-blind
- Eligibility: Safety, Representativeness
- Sample size: Liberal assumptions
- Outcomes: Many primary outcomes
- Analyses: Responders-only
- *Phase III* efficacy trial rare





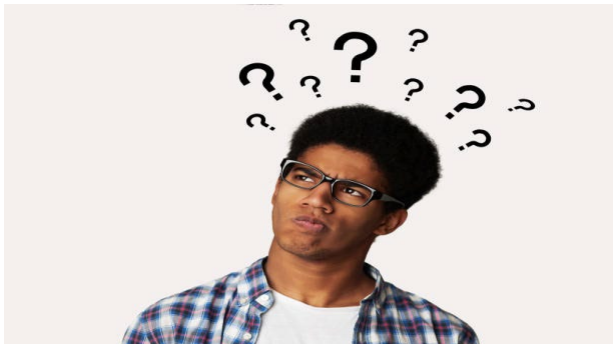
## “Rules” for Randomized Drug Trials

**Goal:** Implementation

**Process:** Multi-disciplinary Teams

**Progression:** Accepted process for progressive development of drug

**Implementation:** Phase III trial window to practice guidelines, 3<sup>rd</sup> party reimbursement, and implementation



Fellows had many questions

## State of the Art in Randomized Behavioral Trials

**Goal:** Discovery

**Process:** Isolation

**Progression:** “One fell swoop” trials

**Implementation:** Implementation gap



Faculty had few answers

# 2004

## Implementation Gap

### Quality of Evidence Needed That a Treatment Works

COUNTRY	RATING SYSTEM	HIGHEST RATING
INTERNATIONAL	GRADE	<i>Phase III</i> Randomized Efficacy Trial
UNITED KINGDOM	Centre for Evidence-Based Medicine	<i>Phase III</i> Randomized Efficacy Trial
SCOTLAND	Intercollegiate Guidelines Network	<i>Phase III</i> Randomized Efficacy Trial
NEW ZEALAND	New Zealand Guidelines Group	<i>Phase III</i> Randomized Efficacy Trial
CANADA	Canadian Hypertension Education Program	<i>Phase III</i> Randomized Efficacy Trial
UNITED STATES	Minnesota Institute Clinical Systems Improvement	<i>Phase III</i> Randomized Efficacy Trial
	American Family Physicians	<i>Phase III</i> Randomized Efficacy Trial
	US Preventive Services Taskforce	<i>Phase III</i> Randomized Efficacy Trial
	American College Cardiology/American Heart Association	Multiple <i>Phase III</i> Randomized Efficacy Trials
	American Academy of Pediatrics	<i>Phase III</i> Randomized Efficacy Trial
	American Academy of Neurology	<i>Phase III</i> Randomized Efficacy Trial
	American College of Chest Physicians	<i>Phase III</i> Randomized Efficacy Trial
	National Comprehensive Cancer Network	<i>Phase III</i> Randomized Efficacy Trial, meta-analyses
	Infectious Disease Society of America	<i>Phase III</i> Randomized Efficacy Trial

# 2024

## SIGNIFICANCE

We Have Guidelines for Identifying Significant Health Problems

### **CURRENT TREATMENT**

- Not very effective
- Uncertain effectiveness (equipoise)
- Effective but not adhered to
- Effective but too costly

### **US PREVENTIVE SERVICES TASK FORCE: GRADE “C”: Cannot Make a Recommendation in Physical Activity Treatments**

- Trials with adequate power and sufficient follow-up
- Culturally appropriate, tailored
- Improve clinician skills in behavioral counseling
- Reduce sedentary behavior

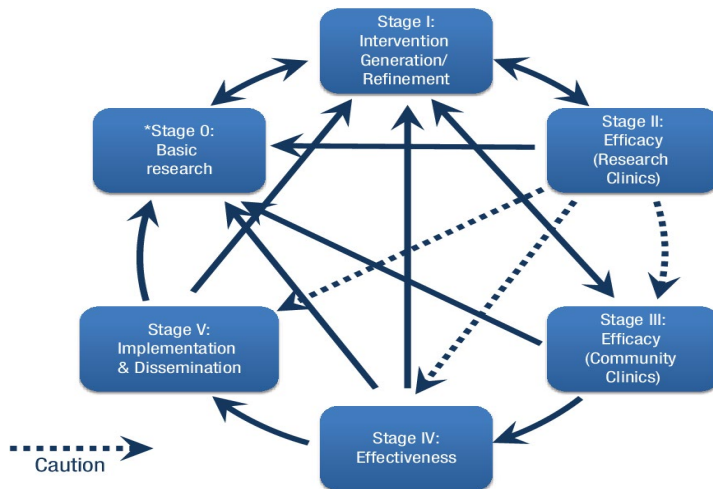


# 2024

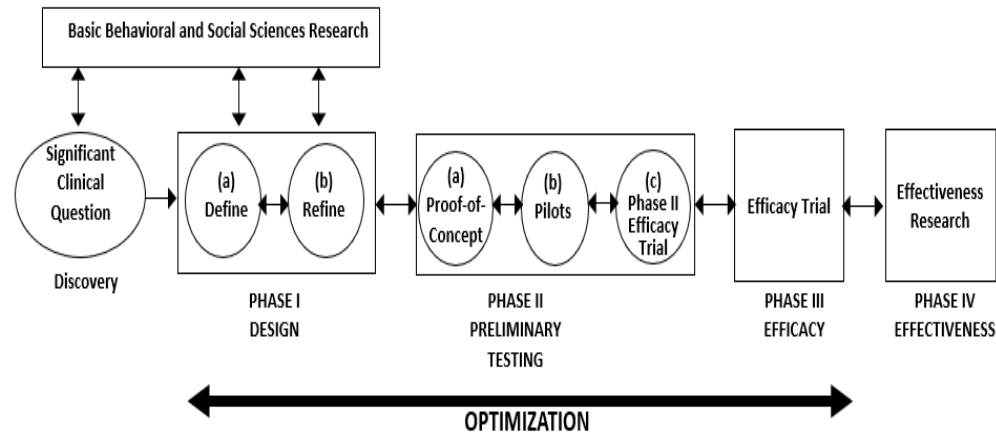
# PROGRESSION

WE HAVE Treatment Development Models

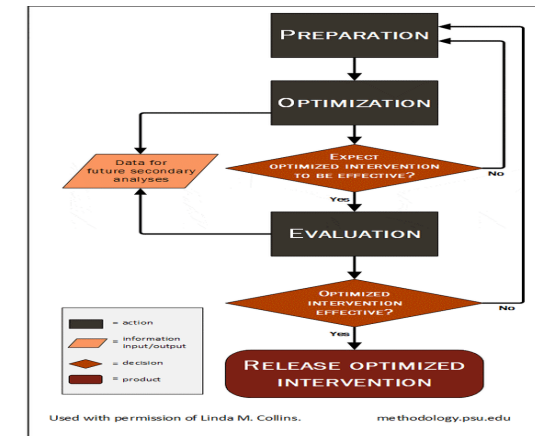
NIH Stage Model



ORBIT Model



MOST



# 2024

## TEAMS

**WE HAVE Networks**



**WE HAVE Training Opportunities  
That Create Teams**



**ORBIT Institute Fellows, 2023**

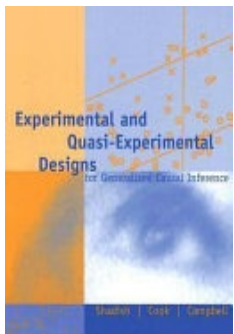
**J Gorzelitz, J Hayes, A Ianoje, A Loughan, S Schrauben**

**Society of Behavioral Medicine 2024**

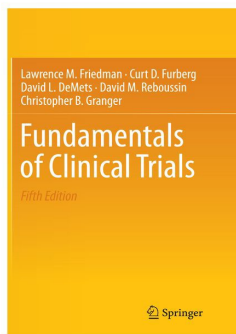
# 2024

## DESIGN

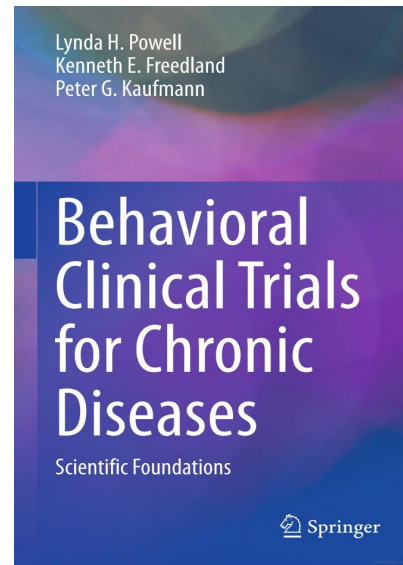
WE HAVE Our Own Book



**Experimental  
Methods**



**Clinical Trial  
Methods**



**Synthesis of Experimental  
and Clinical Trial Methods**

## WE HAVE CONSORT Reporting Guidelines

- non-pharmacological treatments
- eHealth interventions
- pilot/feasibility trials
- template for intervention description
- patient-reported outcomes
- abstract preparation
- non-inferiority designs
- cluster randomized trials
- pragmatic trials
- factorial designs
- data collected during routine practice
- early phase dose finding studies

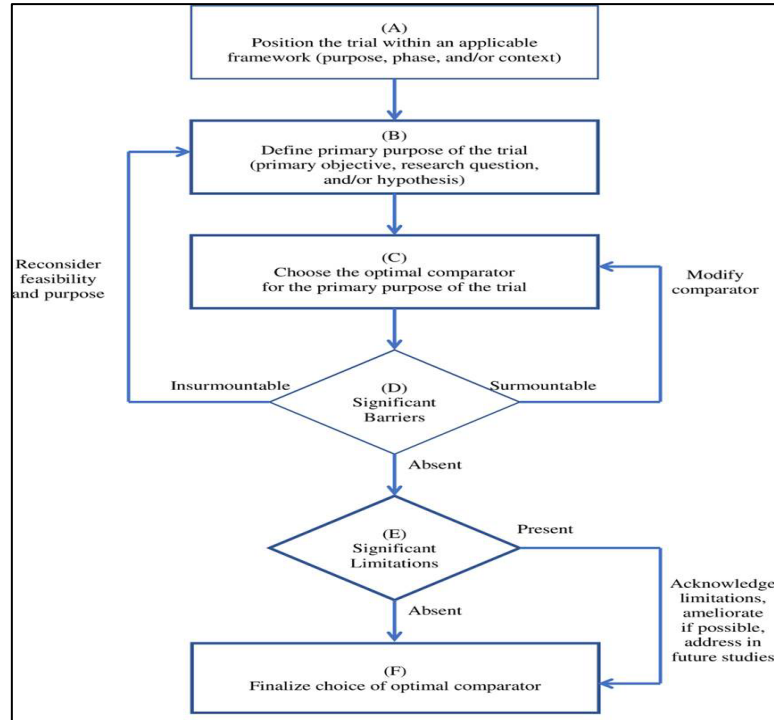
# 2024

## METHODS

### WE HAVE a Meta-Science of Behavioral Trial Methods

#### How to Choose a Comparator

(Freedland et al., 2019)



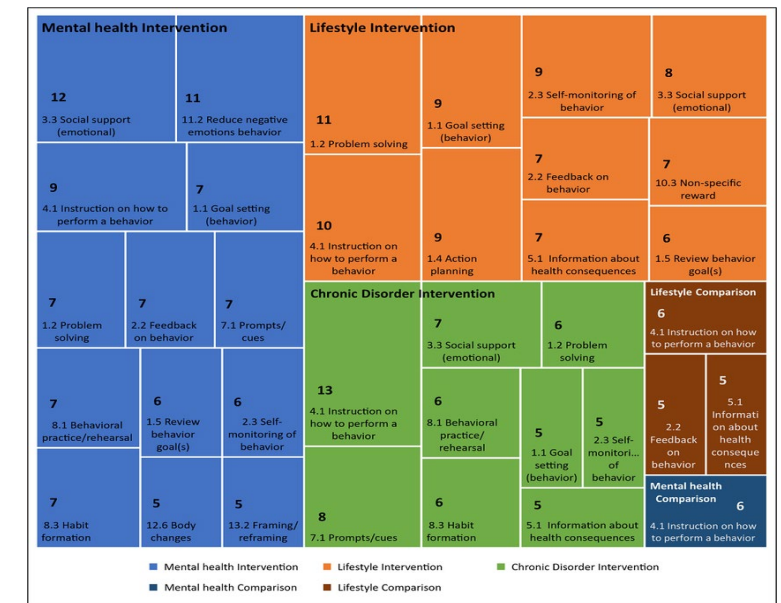
#### How to Do Good Science

(Munafo et al., 2017)

*“Stakeholders of science must not embrace the status quo, but instead pursue self-examination continuously for improvement and self-correction of the scientific process itself.”*

#### How to Describe a Treatment

(Mitchie 2013)



# 2024

# FULLY REMOTE MULTI-SITE TRIALS

WE HAVE *Phase III* Behavioral Efficacy Trials

## BWE The Breast Cancer Weight Loss Trial

**Objective:** Impact of telephone-based weight loss on disease-free survival in overweight or obese women with Stage II-III HER2-negative breast cancer



PI: Jennifer Ligibel, MD

### SPONSOR:

Alliance for Clinical Trials in Oncology:  
--Natl Cancer Institute Trials Network  
--Canadian Clinical Trials Group

### COHORT SITES TREATMENT

3136 Participants  
>150 Sites in US and Canada  
Delivered by phone from central  
coordinating center

# THE FUTURE

*"It is difficult to make predictions,  
especially about the future."*

*Yogi Berra*

## PRECISION LIFESTYLE MEDICINE

### TARGET



Subgroups Most Likely to Benefit:

Eligibility based upon motivation

**True Informed Consent**

*"I understand what you would be asking me  
to do and am ready to do it now."*

**Run-In's**

### TAILOR



Treatment to Patient Characteristics

- Patient Preference
- Patient Baseline Characteristics
- Patient Response

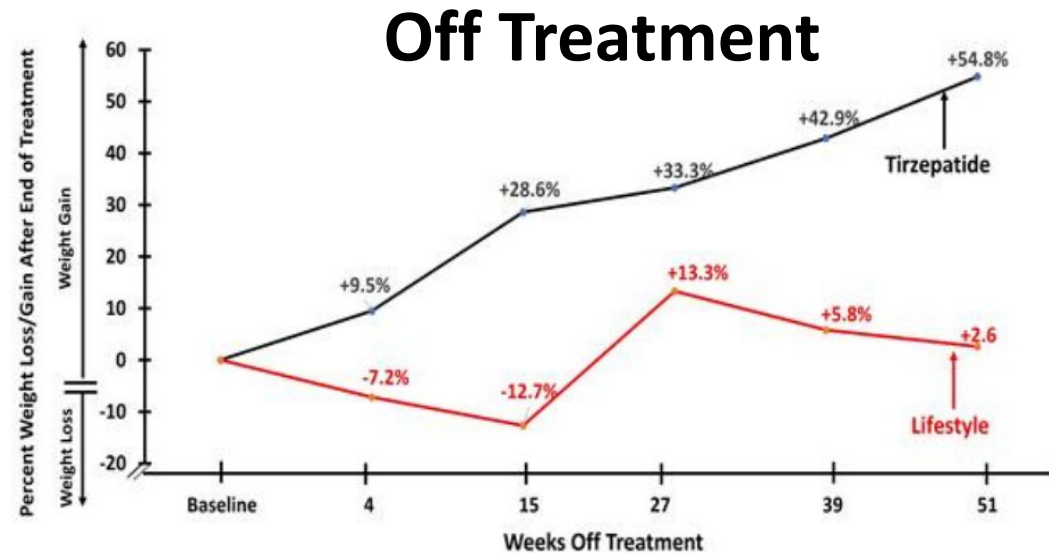
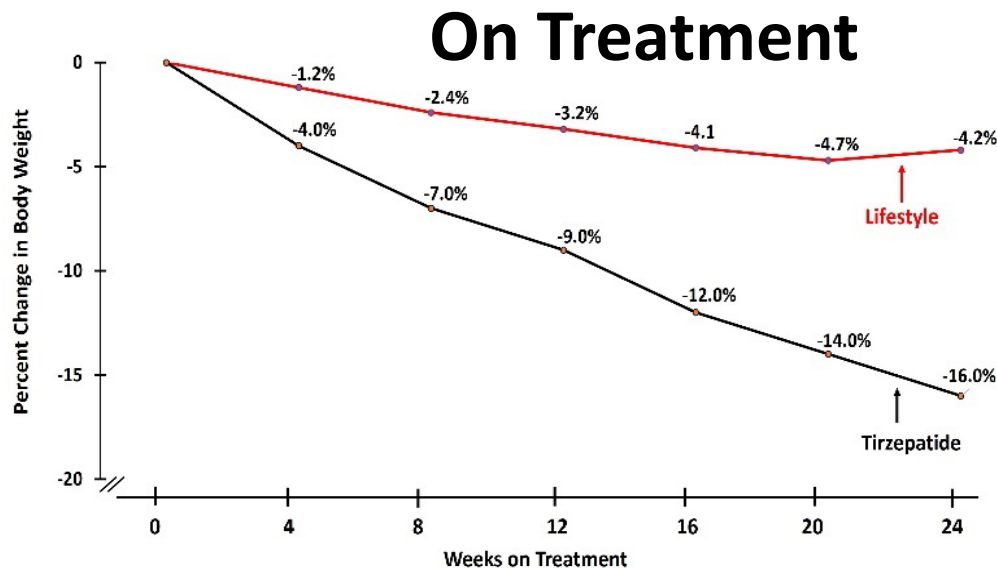


# THE FUTURE

## CROSS-DISCIPLINARY COMPONENTS

Target the individual with behavioral and biological components simultaneously

### WEIGHT LOSS: Lifestyle vs. Obesity Drug



# THE FUTURE

## MULTI-LEVEL INTERVENTIONS

Target individual plus another level of influence simultaneously



**Ecological Model**  
Levels of influence on  
individual behavior

**INDIVIDUAL: Medication Adherence**  
**POLICY: Stable Housing**



**DHOMES**  
Diabetes Homeless  
Medication Support

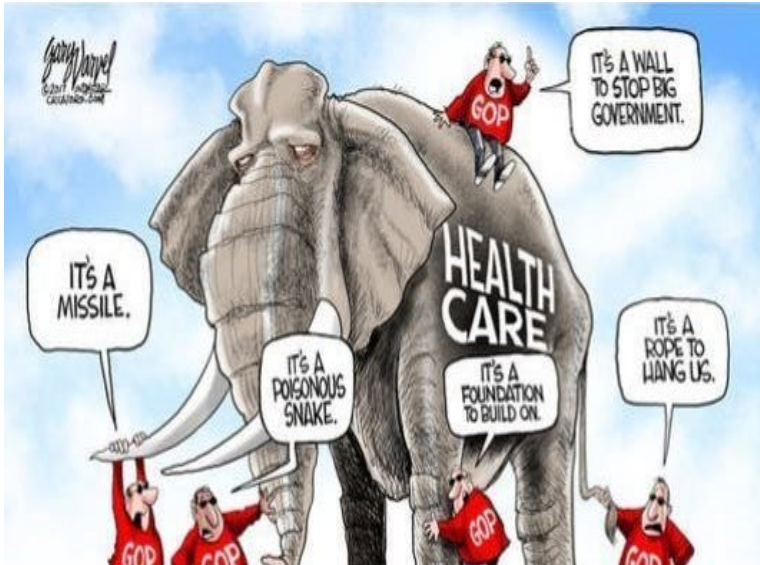
**Behavioral activation for medication adherence  
among people experiencing homelessness**

	<u>Attendance</u>	<u>Acceptability</u>	<u>Final Exam</u>	$\Delta$ <u>A1C</u>
<b>Stable Housing</b>	<b>47</b>	<b>31.8</b>	<b>100%</b>	<b>80%</b>
<b>Unstable Housing</b>	<b>17</b>	<b>14.7</b>	<b>60%</b>	<b>50%</b>

# THE FUTURE

## PLAN FOR IMPLEMENTATION

**Expand List of Stakeholders  
Who Influence Implementation**



**Embed Stakeholder Interests as Outcomes**

**PATIENTS**

**Quality of Life**

**INSURERS**

**Reduction of drug costs**

**INSTITUTIONS**

**Patient Satisfaction**

**COMMUNITY**

**Cost**

# THE FUTURE

## INTERNATIONAL MULTI-SITE BEHAVIORAL TRIAL

SPONSORS	--International Behavioral Trials Network --US Office of Behavioral and Social Sciences Research
COHORT	<b>3500</b> Participants
SITES	<b>25</b> Countries
TREATMENT	<b>Fully remote</b> treatment



Principal Investigators

Kim Lavoie, **PhD**

Simon Bacon, **PhD**



**Where you're headed  
is more important than  
how fast you're going.**

**Stephen Covey**

