



# Biostatistics and Study Design Core

## Co-Chairs:

- Patrick Heagerty, PhD
- Elizabeth L. Turner, PhD



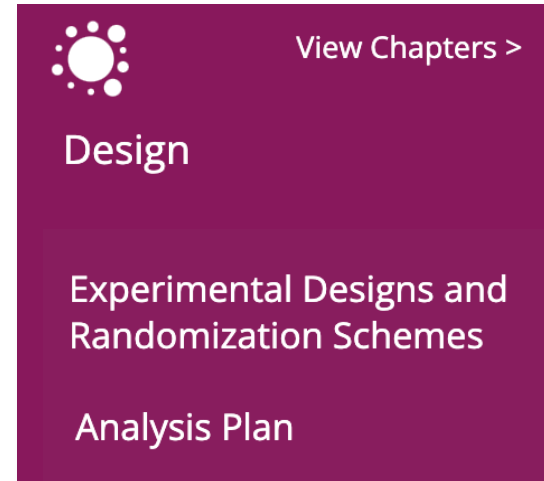
## Mission

- Provide expertise in novel designs and methods for ePCTs
- Document new statistical issues and share knowledge
- Develop methods to address challenges

# Biostatistics and Study Design Core: Key Resources



- Living Textbook chapters on study designs and analysis plans
- Study Design eLearning and video modules
- Intraclass Correlation Coefficient Cheat Sheet
- Design and Analysis of ePCTs workshop (recording available)
- Substantial literature on design and analysis of group-randomized trials



Review Article

## Mixed-effects models for the design and analysis of stepped wedge cluster randomized trials: An overview

Fan Li<sup>1,2</sup>, James P Hughes<sup>3</sup>, Karla Hemming<sup>4</sup>,  
Monica Taljaard<sup>5</sup>, Edward R. Melnick<sup>6</sup> and Patrick J Heagerty<sup>3</sup>



Contemporary Clinical Trials

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Two weights make a wrong:  
Cluster randomized trials with  
variable cluster sizes and  
heterogeneous treatment effects

Xueqi Wang<sup>a</sup>, Elizabeth L. Turner<sup>a</sup>, Fan Li<sup>b</sup>, Rui Wang<sup>c,d</sup>,  
Jonathan Moyer<sup>e</sup>, Andrea J. Cook<sup>f,g</sup>, David M. Murray<sup>e</sup>,  
Patrick J. Heagerty<sup>g</sup>

# Biostatistics and Study Design Core: Contributions to Trials



- Direct impact on many trials, resulting in revised:
  - Statistical analysis plans
  - Sample sizes
  - Trial designs
- Expertise in:
  - Cluster randomization
  - Stepped-wedge designs
  - Handling missing data
  - Heterogenous treatment effects
  - Power and sample size

“Take the Biostats Core Working Group advice seriously—get it early and act on it early.”

—*Ted Melnick, PI of EMBED*

“They helped with our early problems, and we got quite a bit of help towards the end for the publication in *JAMA Internal Medicine*, which was very productive.”

—*Bev Green, Co-PI of STOP CRC*

“Originally...the statistical analysis we used had the effect of overcorrecting our data.”

—*Gloria Coronado, Co-PI of STOP CRC*

# 1:1 Trial Consultations



- CC will schedule within next 3 months to:
  - Discuss your study design and analysis plan
  - Offer expertise and guidance
  - Anticipate and troubleshoot issues
  - Answer any of your questions

