



Health Care Systems Research Collaboratory

Biostatistics and Study Design Working Group Report

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Biostatistics and Study Design Working Group

- Monthly group calls (17 members)
- Bi-weekly core group calls (Liz DeLong, David Murray, Lingling Li, Andrea Cook)

Toolkit and Information Sheets

- **Designing Multi-center Cluster Randomized Trials: An Introductory Toolkit** was originally developed in March 2008 (funded by the NIH, under Contract No. HHSN268200425212C, "Re-Engineering the Clinical Research Enterprise.")
- Its content was updated in September 2013 (Elizabeth DeLong, David Murray, Andrea Cook, Jeremy Sugarman, Lingling Li, Barry Carter, Erik Larson)
- Guidance documents ("information sheets") have been developed by the working group: 1) Varying cluster sizes; 2) ICC; 3) Pair matching versus stratification; 4) Constrained randomization.
- Links to information sheets have been included in the toolkit

Methodological Research

- Fan Li (Master of Biostatistics student at Duke) studied properties of constrained randomization for CRTs
- Conducted simulation studies to investigate treatment effect estimator when
 - a. Comparing simple versus constrained randomization (CR)
 - b. Varying the size of the candidate set for CR
 - c. Varying the number of covariates balanced in CR
 - d. Comparing unadjusted and adjusted models under CR
- Members of the working group have contributed thoughtful discussion and helped direct research
- Study results have been summarized – next step is to write the manuscript

Conference Abstracts

- **Joint Statistical Meetings (JSM) 2014 (accepted)**

“Comparing the Statistical Performance of Conditional F-test between Constrained Randomization and Simple Randomization to Evaluate Treatment Effect in Cluster Randomized Trials - A Simulation Study” (Fan Li)

- **ASCP 2014 (accepted)**

“NIMH/NCCAM Panel: Conducting Pragmatic Trials in Mental Health: Lessons Learned from the NIH Health Care Systems Research Collaboratory” (Liz DeLong, Greg Simon)

- **DIA 2014 (rejected)**

“NIH Health Care Systems Research Collaboratory Biostatistics and Study Design Workgroup”

Ongoing and Future Work

- Working on a manuscript outlining issues in current ongoing UH3 pragmatic trials. Each statistician who wants to participate will submit one statistical and one data issue paragraph highlighted by their trial's design. Target October 2014 for paper submission.
- PCORI methods grant (led by Andrea Cook and Patrick Heagerty).