Small Sample Methodology: Tools for Conducting Intervention Research with Small Samples

Chair: Kathy Etz, PhD, National Institute on Drug Abuse

Co-chair and Presenter: David B. Henry, PhD, Professor, Institute for Health Research and Policy, School of Public Health and Department of Psychology, University of Illinois at Chicago

Presenters:

Donald Hedeker, PhD, Professor of Biostatistics, School of Public Health, University of Illinois at Chicago

Linda Collins, PhD, Senior Researcher, Chapin Hall at The University of Chicago

John Creswell, PhD, Professor, Educational Psychology, University of Nebraska-Lincoln

Purpose of the workshop, including specific learning objectives

Although larger samples are desirable for reasons of statistical power, much prevention research is conducted in situations where organizational or cultural constraints make large sample sizes prohibitive. Because of the inherent challenges of prevention research with small samples, it is important to develop a body of methodological and statistical knowledge that is applicable to these situations.

This workshop will consider some of the issues involved in prevention research with small samples and will focus on four strategies to address the challenges. It will include both quantitative and qualitative approaches, as follows:

David Henry will address methods for increasing the sensitivity of a study and power for detecting true preventive effects. Studies with small samples may be underpowered not only because of the number of participants, but also due to levels of analysis, contamination, selection/attrition/perverse incentives, constraints on design, implementation problems, and/or sensitivity of measures to change. This session will suggest some strategies for addressing each of these threats. Examples of such strategies include randomizing at the individual level, randomizing start times rather than individuals, matching prior to randomization, using a placebo intervention rather than a no-intervention control, estimating expectation using multiple baselines or population data, increasing sensitivity of measures to change, and adding ethnography to the statistics.

Donald Hedeker will present a session on models for clustered, ordinal data, that may be more appropriate for prevention outcomes than are models that assume continuous measurement and multivariate normality. Prevention outcomes often have distributions that make them
inappropriate for most popular statistical models, and are more appropriately considered ordinal outcomes. Despite this, how ordinal outcomes can modeled is often not well understood. Attendees will learn ways to analyze longitudinal and clustered ordinal outcomes, including the proportional odds regression model for ordinal outcomes, which assumes that the covariate effects are the same across the levels of the ordinal outcome. The presentation will cover how to test this assumption and what to do if it is violated. Examples and computer syntax will be given so that attendees can immediately apply these methods to their own data. The presentation will also discuss the statistical power of these methods.

Linda Collins will provide instruction on using simulations in power analysis. Simulation is a particularly useful tool for investigators estimating power with smaller samples and ordinal outcomes. This presentation will demonstrate that simulations can provide power estimates that are more closely tailored to the specific analyses proposed than are possible with even the best “canned” programs for estimating statistical power. This session will begin with a review of the concept of power and its importance for determination of appropriate sample size(s) for a study. A simulation involving a standard hypothesis test for a difference between proportions using samples from two binomial populations will be examined. The results will be compared to power calculations using formulas appropriate for large samples. Following this foundation, participants will learn how to use simulation to conduct power analyses for a variety of designs. Demonstrations of sample size determination for means comparisons and regression models will be included, and other models will be explored as time permits. Computer code used to carry out simulations and power calculations will be provided to participants after the session.

John Creswell will discuss best practices in mixed methods research, considering mixed methods in small samples. He will address the basic characteristics and major designs in mixed methods research, review necessary skills for conducting mixed methods research and present guidelines for proposal development and reviews drawing from a recent document developed for the NIH OBSSR entitled "Best practices in mixed methods."

**Target workshop audience**

The targeted workshop audience is individuals at any professional stage who are working with, or contemplating working with, small samples. At least minor familiarity with statistical concepts will be helpful as background including familiarity with the basic process of hypothesis testing. The concepts of p-value and significance will also be reviewed.

**Materials to be provided to attendees**

Attendees will be provided with handouts of all presentations and, as described above. In addition, computer syntax will be provided in two of the sessions.

**Presenters**

Dr. Etz is a program official at the National Institute on Drug Abuse and coordinates American Indian and Alaska Native activities there, research that is often characterized by small samples.

Dr. Henry has worked with the Center for Alaska Native Health Research for many years and has applied the techniques that will be discussed to small samples. He has experience in conducting workshops on small sample methodology, including a recent two-day workshop in Fairbanks, Alaska.
Dr. Hedeker is a nationally recognized expert in mixed models and has co-authored a recently published book on this topic. He has conducted many similar workshops (see invited talks section of his CV) including pre-conference workshops for SPR in the past. Note, however, that this topic is distinct from his past SPR workshop.

Dr. Collins has made presentations on accurately estimating statistical power internationally and is recognized as an expert in this area.

Dr. Creswell recently served on a team to develop the NIH publication Best Practices for Mixed Methods Research in the Health Sciences. He has co-authored 19 books, many of which focus on research design, qualitative research, and mixed methods research. In addition, he co-directs the Office of Qualitative and Mixed Methods Research at the University of Nebraska. He founded and served as Co-Editor for the Sage journal, Journal of Mixed Methods Research.

Workshop Outline

- Orientation to Workshop
- Talk 1 (one hour and 45 minutes) Overview of small sample research and relevant Issues – David Henry
- Talk 2 (one hour and 45 minutes) Accurately Estimating Power for Small Samples– Linda Collins
- Talk 3 (one hour and 45 minutes) Mixed Models for Ordinal Data – Don Hedeker
- Talk 4 –(one hour and 45 minutes) Mixed Methodology in Small Sample Research – John Creswell

Instruction will not be hands on – however, computer code will be provided in the second and third talks.