

# Organization of Workshop on Improving Health Research for Small Populations

## Brief Statement of Task

A National Academies of Sciences, Engineering, and Medicine ad hoc planning committee was convened to organize a public workshop to discuss the methodological challenges of conducting research on small, underrepresented population subgroups in health research. The workshop will consider ways of addressing the challenges of conducting epidemiological studies or intervention research with small population groups, including alternative study designs, innovative methodologies for data collection, and innovative statistical techniques for analysis. Specifically, the workshop will address approaches for identification, recruitment, and retention of study participants to maximize the sample sizes of small groups in research studies; epidemiological design and analytics approaches for small samples; and intervention study design and analytic approaches for subpopulations.

This workshop is sponsored by the National Cancer Institute (NCI), the National Institute on Minority Health and Disparities (NIMHD), and the Office of Behavioral and Social Sciences Research (OBSSR) of the National Institutes of Health, and the Robert Wood Johnson Foundation.

## Organization of Workshop

Public health *surveillance* is generally accomplished through observational studies of the health status and health needs of population groups, either using existing data sources or designing surveys to collect needed information. Examining the effects of an *intervention* requires a carefully designed study that may be referred to as an intervention, prevention, behavioral study, or clinical trial.

The panel has chosen to structure the workshop around the surveillance/intervention stages in health research. Sessions 2 and 3 focus on data for observational studies—using available data, or collecting new data; and sessions 4, 5, and 6 focusing on various aspects of intervention trials—study design, recruitment and intervention, and analysis.

**Session 1** sets the tone for the workshop by describing real challenges in health research for small populations.

**Session 2** provides information about several of the challenges and new approaches to using available data (government or other surveys, administrative records, electronic health records, and geospatial techniques.) This is very important especially for larger populations that are well represented in existing data sets.

**Session 3** can be viewed as identifying what the survey research community knows about finding people for the purpose of getting data on small populations. The discussion includes issues related to surveying populations for which a sampling frame can be constructed, and using probability sampling to produce estimates that are generalizable to the desired small population. It also highlights approaches to surveying “hidden” populations—those for which no sampling frame can be constructed. While these approaches still allow researchers to learn about those surveyed, the results are typically not generalizable to the population. Issues of generalizability, as well as what can be learned and what cannot be learned from these methods, will be discussed.

**Session 4** provides an introduction to some of the new and emerging designs that are most relevant for small populations/samples, and discusses the situations in which each might be most useful. The session goes on to provide examples of applications of these interesting new designs and mixed methods approaches, along with concluding discussion of guidelines and decision rules for their use.

The randomized controlled trial (RCT) has become the gold standard in health intervention research. The challenges, especially with small populations or small samples, include navigating logistical and ethical issues that can arise, and ensuring that data from RCTs are managed in a way that will not compromise power and external validity. Recent alternative designs make use of optimization strategies to more efficiently use available information to maximize power with modest sample size. Other mixed methods approaches blend qualitative and quantitative approaches.

**Session 5** starts with an overview of challenges to recruitment and retention. It goes on to describe solutions through organization and community and the use of technology. In addition to designing a study that can provide data for small populations, researchers must be successful in recruiting eligible participants and retaining them for the duration of the study. While the process is no different for small population research, recruitment and retention are even more important when losing study participants can easily kill a study.

**Session 6** includes discussion of approaches for maximizing the yield and power of a study, and a discussion of the potential for the use of Bayesian methods to improve analysis of small sample studies. The third presentation describes a different, but important analytical problem: that of estimating the population size of a “hidden” population. Though the design of a study and the analysis go hand-in-hand and effective sample size is directly related to the success of recruitment and retention, there are some analysis considerations and techniques that can improve the power or yield of a study with a small sample.

**Session 7** is a panel discussion summarizing the workshop, what has been learned, and identifying next steps.