What is a small population?

Depending on the context, "small" populations can be identified in several ways. One definition of a "small" population sets a specific count and considers a population to be small if its size falls below that number. In other cases population size is small, fixed, and readily identifiable; for example, the National Aeronautics and Space Administration commissioned the Institute of Medicine (IOM) to comment on issues and challenges associated with small clinical trials focused on astronauts (IOM 2001). Here the denominator was known through the precise number of people entering the profession. IOM (2001, p. 6) expands this framework to other situations that might warrant a small clinical trial (where "small" means a reduced number of participants) including trials based on:

- Rare diseases
- Unique study populations (e.g. astronauts)
- Individually tailored therapies
- Environments that are isolated
- Emergency situations
- Public health urgency
- Restricted resources coupled with an important need."

In other instances, however, relying on a fixed number is not an effective way to determine if a population is small. Often it depends on how the population is perceived in relation to a larger group. In his monograph on hard-to-survey populations, Tourangeau et al. (2014, p 4) noted that "problems [in sampling] arise when a target population represents a small fraction of the frame population" and observed that Chapter 19 of the monograph "distinguishes major subgroups or domains (constituting more than 10% of the total population), from minor subgroups (1 to 10 percent) and from mini-subgroups (less than 1% of the total population)"

Much of the interest in studying health disparities for "small" populations was stimulated by the Department of Health and Human Service's Healthy People project[1] in 2010. The project, which set a vision and strategy for improved health outcomes by 2020, listed as one of their goals a desire to "achieve health equity, eliminate disparities, and improve the health of all groups." The elimination of these disparities and inequity is to be assessed across the following categories: race/ethnicity; gender; socioeconomic status; disability status; lesbian, gay bisexual, and transgender status; and geography. Several of these categories—in particular, race, LGBT status, and even some geographies—can be indicative of small populations, using the aforementioned definitions.

The National Cancer Institute (NCI)[2] and HRSA[3] both provide a wealth of information about measuring diversity of health outcomes and/or lack of access to health care. Theoretically, any

[1] https://www.healthypeople.gov/sites/default/files/HP2020_brochure_with_LHI_508_FNL.pdf (December 2017)
[2] https://www.cancer.gov/about-nci/organization/crchd/about-health-disparities (December 2017)
[3] https://bhw.hrsa.gov/shortage-designation/muap (December 2017)

population can be used as a point of reference to determine the extent of a health disparity in a subgroup of that population. However, for the purpose of this workshop we consider when "small population" matters. This definition implies a small group of theoretical interest for some specific health related reason. This could be a group that might react to a given treatment in an unusual way (e.g., the effect of a drug on a group possessing a novel allele), or it could be people whose work exposes them to a particular hazard, such as miners in a particular community). Small populations can also occur from combination of characteristics. As such, the range of possibilities is limitless and is contingent on a researcher's specification of a specific research question with a specific group, e.g., members of small American Indian and Alaska Native tribal groups; "undocumented" immigrants; small religious groups such as the Amish; sexually abused children or adults; men who have sex with men, sex workers, and others at risk for HIV; illicit drug users; children in severe poverty; and the homeless mentally ill.

These small populations are often also hard-to-reach, both because of the difficulties sampling them and at times, out of negative histories with social institutions and with past research, their unwillingness to participate in research studies. Tourangeau et al. (2014) listed many examples of the challenges with surveying these groups and possible methods that may be used. The monograph provides a useful framework for distinguishing hard-to-survey populations as:

> "populations that are hard to sample, those whose members are hard to identify, those that are hard to find or contact, those who are hard to persuade to take part, and those willing to take part but hard to interview. These distinctions reflect the main steps in many surveys, beginning with sampling and ending with data collection."

Populations that are hard-to-reach and hard-to-survey are likely to also be hard-to-study for health research. Often, "hard-to-study" is another attribute defining what we mean by "small population." A synonym for "hard" is "expensive." When lower-cost groups are readily available, funding constraints can lead small populations to be overlooked.

Some of the difficulty in defining a small population stems from difficulty in defining the "population" at large. A useful study design would target individuals from a well-defined group from which participants could be recruited into an intervention that would yield results generalizable to others in the group. This is a useful goal, but requires that members of the group are similar enough in terms of characteristics related to the outcome of interest and the effectiveness of study approaches. Even groups that fall into a particular classification may be ethno-culturally distinct. This cultural distinctiveness can require adapted or culturally grounded intervention for the group, requiring small sample intervention research to test if the new intervention is effective. This is typically the case, for example, for an American Indian, Alaska Native, and Native Hawaiian group, or for a rural Scotch-Irish Appalachian population.

Context can be important for shaping health outcomes in a number of ways, for example exposure to toxic chemicals in the air, or residence in a neighborhood where extreme poverty shapes access to food, education, and employment. In many cases, the spatial extent of the context that matters for health will be very small--for example when exposure to lead increases dramatically near busy roads or old houses. This means that researchers have to think carefully about the extent that matters for contextual effects they may wish to include in their research.

Research questions of large importance to health disparities and ethnic minority health research

may involve small samples because of the size of the distinct population that is affected. However small populations are defined, intervention studies with these groups will likely necessitate small sample research. Study designs are often underpowered due to their sample sizes. As noted by Fok, et al. (2015),

> "It is therefore tempting to define "small" merely in terms of statistical power. However, lack of power may result from weak effects as much as from sample size. A sample size that is adequate for a medication study with strong effects may be insufficient for a psychosocial prevention trial with more modest effect sizes."

However, common to the many definitions of "small" is a shared concern regarding the extent to which individual observations have influence on the results of an analysis; in small samples, influence of outlying observation is amplified, reducing representativeness.

References

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