Collecting Comprehensive and Representative Input

HYPOTHETICAL SCENARIO: QUALITATIVE RESEARCH USING A SINGLE FOCUS GROUP

Disclaimer: This hypothetical scenario provides practical supplemental information to illustrate some important concepts presented in FDA's draft guidance on Patient-Focused Drug Development: Collecting Comprehensive and Representative Input, which the Agency displayed for public comment on June 12, 2018. This hypothetical scenario should not be construed as formal advice from FDA. FDA encourages stakeholders who are considering collecting and submitting patient experience data to have early interactions with FDA. As the science of patient input matures, or in response to comments received on FDA's guidance, the scenario may be updated. The scenario focuses on a specific methodological issue and does not address all aspects of good research design, conduct, analysis, reporting, data protection, and patient privacy, including all potential legal obligations.

SITUATION

An academic center is conducting qualitative research to support development of a new patient-reported outcome (PRO) instrument for a disease affecting 10,000 people in the U.S. Symptomatic onset and diagnosis of this disease typically occur in adulthood, and symptom manifestations and severity vary widely among patients. This research is being conducted in the pre-competitive space, that is, it is not associated with any particular medical product development program. The researchers hope that an eventual PRO instrument may be able to broadly support medical product development for this disease area in the future.

Funding resources are limited, and the researchers plan to conduct one, in-person focus group held at the academic center's location. The researchers have proposed that this focus group will constitute a sufficiently representative sample because it will include adult patients from across the U.S. who travel to the center for treatment. (The researchers note that there are a limited number of academic or medical centers that specialize in the treatment of this disease.) The researchers also state that they plan to confirm the content of the instrument and develop items via quantitative studies with a much larger and more geographically diverse sample.

A POTENTIAL CONCERN FOR MEDICAL PRODUCT DEVELOPMENT

Focus Group Composition

Determining the adequate number and geographic diversity of focus groups for the purposes of developing a PRO instrument depends on a number of factors, including the size and demographic characteristics of the patient population, as well as the scientific understanding of the natural history of the disease. In this hypothetical situation (and without any other information), the

researchers' plan would raise concern that the single focus group does not comprise a sufficiently heterogeneous sample of the patient population. Even though participants live in different geographic areas, there may be important systematic differences (such as in disease severity, symptom manifestations, or socioeconomic status) between patients who travel to another city/center for their medical care and those who do not.

Although a follow-up quantitative study is planned, if the original qualitative research does not sufficiently reflect the heterogeneous experiences of the patient population, the follow-up quantitative research risks failing to meet its objectives. For example, there may be a sizable subset of the population who experience an important sign or symptom of disease that was not captured in the focus group. In this case, the sign or symptom may not be suitably considered in the quantitative study. There may also be important differences in the language that patients use to describe their symptoms. Ultimately, limited qualitative research may result in a PRO instrument (and the related study endpoint) that is used in future clinical trials, but that may not be adequate to assess the efficacy of the study drug.

HOW COULD RESEARCHERS ENHANCE THE RESEARCH EFFORT?

Understanding that the optimal enhancements depend on a number of factors, there are a few general, cost-effective ways, that can often be used complementarily, to increase the geographic and demographic representation of focus groups. The suggestions below do not represent an exhaustive list of options.

Collaborate with partners

Collaborate with patient groups, other researchers, clinicians, and/or recruiting organizations who can help identify a wider set of potential participants, provide facility space, or moderate focus groups in different geographical areas, using the same focus group protocol and facilitation guide.

Engage with patients virtually

Consider conducting virtual focus groups or individual interviews through internet-based videoconferencing or telephone. The in-person focus group protocol, procedures, and analysis may require some modification to work well for a virtual setting, keeping in mind logistical and resource requirements, such as time zones, computer system compatibility, ease of use for participants, and recording capacity. If multiple research methods are used, the researchers should provide a separate analysis for each method in their study report.

FOR MORE INFORMATION:

www.fda.gov/FDAgov/Drugs/DevelopmentApprovalProcess/ucm610279