Patient Experience Data Should Influence Trial Design, FDA Guidance Says

New recommendations for the collection, reporting, management and analysis of patient experience data to be used in trial design are outlined in a final guidance the FDA released last week.

An eight-step process for collecting and using patient experience data set out in the guidance includes determining the population from which to collect data, considering the research setting and data collection instruments, and deciding what analyses of the data will be needed to achieve research objectives. In this process, trials should examine previous studies and relevant research literature and consult subject matter experts to help determine the most appropriate questions to ask patients.

The guidance outlines how to identify target patient populations and provides recommended methods of collecting data. It is largely unchanged from the June 2018 draft guidance with one exception: the FDA decided to delete the section on qualitative and quantitative research methods, an agency spokesman said, choosing to address that issue in a later guidance.

Sampling methods and their potential limitations are also covered as well as factors to consider to ensure the data collected is representative of the total patient population. Acceptable data collection methods described in the guidance include interviews, focus groups, facilitated discussions at meetings, observational studies, document review, survey instruments, audiovisual materials, digital health technology, and social media and verified patient communities. According to the guidance, patient experience data should address how signs and symptoms of a disease affect a patient’s day-to-day functioning and quality of life, changes in symptoms over time, symptoms and burdens related to treatment, and how patients view the potential tradeoffs between treatment benefits and risks.

The guidance is the first of four developed for the agency’s Patient-Focused Drug Development (PFDD) initiative. Guidance Two, which was released in draft in October 2019, will discuss what questions to ask and how to develop non-leading questions that will be well-understood by a wide range of patients and other stakeholders.

A third guidance, still in the planning stage, will cover what and how to measure input that can be used to develop fit-for-purpose clinical outcomes assessments (COA) that focus on results that are important to patients. The last guidance will explain how to incorporate a given COA tool or set of measures into a defined clinical study endpoint and how to define meaningful change in that endpoint.

Steve Smith, WCG Clinical’s president of patient advocacy, said sites and sponsors would be very wise to become familiar with this guidance. To implement it, they should start by forming an alliance with patient communities that can help them formulate the questions they should ask patients. In some cases, this input could help start a trial that wouldn’t be possible otherwise, he says.

To read the guidance, click here: https://bit.ly/2V1dNJX.

-By Leslie Ramsey
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