Revolutionizing Clinical Trials: The Power Of Patient-Focused Drug Development

By Medical Research Network

Incorporating patients' perspectives in drug development has, paradoxically, been historically uneven, with many programs and trials far more focused on clinical endpoints, regulatory considerations, and technical challenges. This paradigm is rapidly shifting, as patient-focused drug development (PFDD) becomes an increasingly important component of clinical trial design and execution. These trends have been largely driven by a need to improve recruitment and retention, as well as by recognition from stakeholders that a more holistic approach to clinical studies — one that accounts for patient experience and engagement — can serve to improve nearly every aspect of trial conduct.

The Food and Drug Administration (FDA) has collaborated extensively with patient advocacy groups, sponsors, and key stakeholders on outlining the best strategies for facilitating patient-forward clinical trials. The result of these discussions, the FDA's four-part series on PFDD,[1] offers a roadmap for organizations working toward incorporating more patient-centric frameworks in their trial design.

The first two installments in the series, *Collecting Comprehensive and Representative Input*[2] and *Methods to Identify What is Important to Patients*,[3] have been finalized, while the third guidance document, *Selecting, Developing or Modifying Fit-for-Purpose Clinical Outcomes Assessments*,[4] and the fourth, *Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision Making*,5 have been shared as draft guidance.

Each document attempts to address how researchers, clinical Healthcare Providers (HCPs), trial subjects, product developers, and other clinical research contributors can collect patient experience data, drill deeper to collect the right data, enable easier data sharing between patients and providers, and better leverage patient data to inform decision-making. Home healthcare solutions can play a pivotal role in this transformation by offering a bridge between clinical trials and real-world patient experiences.

The following is a breakdown of key takeaways from each guidance document, including insight into how home healthcare solutions can help sponsors achieve many of the core goals of PFDD:

PFDD Guidance 1: Collecting Comprehensive And Representative Input

In the first installation of its guidance series, the FDA provides a framework for patient engagement that can help stakeholders structure meaningful interactions with participants, enabling them to capture more valuable insights and establish patient-reported outcome (PRO) measures that more accurately reflect the impact of a treatment and protocol on patients' lives. This guidance, spurred by the 21st Century Cures Act[6] and a commitment made under the Prescription Drug User Fee Act (PDUFA) VI,[7] addresses methodological approaches to collecting patient experience data, as well as considerations for data collection, reporting, management, and analysis.

According to the Cures Act, this patient experience data is defined as data that is:

- Collected by patients, family members, caregivers, advocacy organizations, research foundations, researchers, drug manufacturers, and/or anyone with approved access to participants.
- Intended to provide information about patients' experiences, including the physical, psychological, and social impacts of a disease or condition as well as the impact of a therapy or clinical investigation on those variables and patient preferences regarding treatment.

This data can be collected throughout the development cycle, from the launch of a discovery program or independent of any specific development program. Qualitative, quantitative, or a combination of methods may be appropriate depending on the goals and structure of a study, and selecting the right patients hinges on defining clear enrollment criteria linked to the research goals or questions to be addressed. Some other key factors to consider include:

- Target population and their availability.
- Most valuable information to be generated through a study.
- Ability to leverage existing literature and data.
- Timeframe or timetable in which a study is to be conducted.
- Projected long- and short-term impacts of the information being gathered.
- Budget constraints.

For patient experience data being collected as part of a clinical trial, patients can often self-report, with or without assistance, on-site or remotely. However, there are situations where, due to factors such as severity of illness, cognitive function, health literacy or fluency, patient data reporting is difficult without assistance from family, caregivers, or advocates. Additionally, for many rare diseases and other highly specialized trials, many participants may be precluded from traveling to an investigator site regularly, or at all, making access to medical personnel or caregivers at home who can help collect this data a valuable resource for trial teams.

PFDD Guidance 2: Methods To Identify What Is Important to Patients

The second installment of the FDA's PFDD guidance details methods for eliciting data from patients; in it, the agency breaks down qualitative, quantitative, and mixed methods, guidance for structuring an approach to data collection based on these different methodologies, and how to conduct data collection concurrently or sequentially when employing mixed method approaches.

The guidance likewise offers insights into how to manage reporting for patients with differing abilities, including how to avoid proxy reporting from caregivers for patients both able and unable to self-report. Having access to home healthcare personnel can be helpful for this, as these providers can assist users with the tools designed to collect this data and help ensure that potential sources of bias from family members or caregivers are controlled.

For many studies, in-person participation across various activities is ideal; the FDA's guidance notes that in-person patient experience data collection "allows for collection of both verbal and nonverbal responses to help inform data interpretation" and enables a wider range of written and brainstorming exercises that can be more easily incorporated in order to better elicit information.It also acknowledges the barriers to in-person data collection, which can include travel costs, facility and space rental fees, and the health or economic constraints affecting patients.

By allowing remote participation in studies through tools such as home healthcare solutions, sponsors and teams can widen their participation pools and improve not only retention, but also data acquisition.

PFDD Guidance 3: Selecting, Developing Or Modifying Fit-For-Purpose Clinical Outcomes Assessments

Clinical outcome assessments (COAs) are crucial tools in medical research, particularly in clinical trials, as they provide a standardized way to measure how a patient feels, functions, or survives as a result of a medical intervention. Guidance 3 delves into the best approaches for "selecting, modifying, developing, and validating" COAs as well as outlining the four primary forms of COAs: PROs, observer-reported outcomes (ObsROs), clinician-reported outcomes (ClinROs), and performance-based outcomes (PerfOs) data.

Findings measured by a well-defined, reliable COA are important for several reasons: measuring what matters to patients is crucial to improving patient-centric drug and device development and design, while ensuring these measurements are clear and accurate arejust as critical in appropriately evaluating the effectiveness, tolerability, and safety of treatments.

In addition to outlining the various types of COAs, describing how to leverage COA scores to establish COA-based endpoints, and detailing how to create "fit-for-purpose" COAs that best serve a study's goals, the document underscores the importance of incorporating patient perspectives in the development of COAs.

COAs can inform healthcare providers' decisions about treatment options, helping to identify treatments that improve patient outcomes and quality of life, in turn leading to better care. Enabling their effective use in clinical trials comes down to both design and deployment — ultimately, access to the right platform technologies and support can make it easier to leverage eCOA, ePRO, and other data interfaces, even in decentralized trial settings.

Solutions like those employed by Medical Research Network (MRN) that combine global trial site networks, home trial support, and eClinical technology platforms can help support improved reliance and reliability for these assessments.

PFDD Guidance 4: Incorporating Clinical Outcome Assessments Into Endpoints For Regulatory Decision Making

The fourth and final installment of FDA's PFDD guidance focuses on detailing the methods, standards, and technologies for collecting and analyzing COA data for regulatory decision-making, with particular attention given to selecting COA-based endpoints and determining clinically significant changes to these endpoints. For endpoint selection, FDA's guidance states that "[…] generally, endpoints that are based on COAs should (1) reflect an aspect of the patient's health that is meaningful; and (2) be capable of supporting an inference of treatment effect within the context of the planned clinical trial."

Guidance 4 offers significant details in how to present COAs in regulatory submissions, how to best validate COAs to ensure they meet both regulatory standards and reflect meaningful patient outcomes, and how incorporating feedback through engaging with patients and stakeholders throughout the COA development process and clinical trial design can ensure their relevancy and effectiveness throughout a study.

The FDA recommends that sponsors measure how a patient's status, as measured by a COA-based endpoint, corresponds to how they feel and function, and how well-calibrated its scoring metrics are in reflecting that correlation.

For example, the document notes "if a treatment is shown to reduce scores on a performance outcome measure by an average of 2 points on a 15-point scale, it would be helpful to know whether a 2-point difference corresponds to something that patients would notice as important in their daily lives."

Knowing how a COA-based endpoint score relates to patient experience and being sure those scores track as closely as possible with patient experience, is crucial to determining the meaningfulness of a COA-based endpoint result.

The document likewise adds that "to demonstrate respect for the patients and/or caregivers who participate and maximize the quality and completeness of information collected in a clinical trial, sponsors should consider ways to minimize the burden of participation and increase the convenience and value of participation to patients and/or caregivers."

Incorporating home trial support services can achieve this in a number of ways, including data collection, enabling improved data quality through the use of unified, integrated platform processes for eCOA.

Conclusions

By embracing PFDD, the pharmaceutical industry is shifting toward a more patient-centric approach to clinical trial design and execution. This paradigm shift is driven by the need to improve patient recruitment and retention, as well as the recognition that a patient-centric approach can enhance the overall quality of clinical trials.

The FDA's four-part PFDD guidance series provides a valuable framework for incorporating patient perspectives into clinical trials by delineating the best practices for collecting comprehensive and representative patient input, identifying what matters most to patients, optimizing COA development and selection, and incorporating COAs into regulatory decision-making.

Home healthcare solutions can play a crucial rolein realizing the goals of PFDD. By facilitating remote participation, assisting with data collection, and providing support to patients, home healthcare providers can help ensure that clinical trials are inclusive, efficient, and patient-centered.

By leveraging home healthcare solutions, sponsors can enhance the quality of patient experience data, improve patient retention, and ultimately accelerate the development of life-saving therapies.

References

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About MRN

MRN accelerates patient recruitment and improves patient engagement and retention through site-centric and patient-centric solutions.

As an innovative market-leader, MRN provides customized solutions to optimize each individual protocol and create more flexible, efficient and accessible clinical trials that deliver accelerated timelines.

Through integrated in-home visit delivery and a vast global network of trained, research ready sites, all empowered by MRN's digital solutions, MRN engages with and empowers diverse communities around the world to participate in and advance medical research.

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