



## Lack of Uptake by Sponsors of the Draft FDA Recommendations for PROs in Oncology



Anthony T. Everhart, MD

Patient-Reported Outcomes (PROs) are essential in capturing the patient experience in oncology trials, providing insights beyond traditional clinical endpoints like tumor response and survival. Despite the clear benefits, the adoption of [PROs in oncology](#) has been slow, with only 8.3% of oncology drugs approved by the FDA between 2010 and 2020 including PRO-related labeling.

In June 2021, the FDA released draft guidance titled “[Core Patient-Reported Outcomes in Cancer Clinical Trials](#)” to enhance the quality and consistency of PRO data. However, the uptake of these recommendations by pharmaceutical sponsors has been limited.

This blog post examines the challenges pharmaceutical companies face in implementing PROs in cancer clinical trials as outlined in the FDA's draft guidance and suggests ways to overcome these obstacles.

### The Value of Patient-Reported Outcomes in Oncology

PROs allow patients to directly report their symptoms, functional status, and overall quality of life, providing a more holistic view of treatment impacts. They are vital for understanding how cancer treatments affect patients' daily lives, helping researchers to make informed decisions that prioritize patient well-being.

Including PRO data in drug labeling can aid both patients and prescribing physicians in weighing the risks and benefits of treatments, offering critical insights that go [beyond traditional measures of efficacy](#).

### Overview of the FDA's Draft Guidance on PROs in Oncology Trials

The FDA's draft guidance, “Core Patient-Reported Outcomes in Cancer Clinical Trials,” aims to standardize the collection and use of [PRO data in oncology trials](#). By focusing on a core set of PROs, such as disease-related symptoms, symptomatic adverse events, physical function, and overall side effect impact, the guidance seeks to improve the consistency and comparability of PRO data across trials.

Additionally, it recommends aligning PRO assessments with treatment schedules of the investigation's products, conducting baseline assessments, and increasing the frequency of assessments during the initial treatment phases. The guidance also emphasizes the importance of collecting data on missing assessments and suggests using electronic PROs to reduce patient burden and improve data quality.

### Challenges Faced by Pharmaceutical Companies in Implementing PROs

Despite the FDA's efforts to promote the use of PROs, several challenges hinder widespread adoption among pharmaceutical companies:

**Operational Complexity** - Implementing PRO assessments in clinical trials requires additional resources, such as validated questionnaires, data collection tools, and trained personnel. The associated costs, logistical complexities, and lack of internal expertise can make companies hesitant to fully embrace PROs.

**Lack of Standardization** - Although the FDA's draft guidance provides a core set of PROs, there is still significant variability across cancer types and treatments. The existing diversity of PRO measures complicates the standardization process, and pharmaceutical companies often struggle to harmonize PRO assessments across different trials.

**Regulatory Uncertainty** - Pharmaceutical companies may fear being early adopters of the draft guidance, concerned that the FDA may not accept PRO data for regulatory submissions despite adherence to the recommendations. Balancing PRO data collection with other complex trial requirements can also be challenging, creating a barrier to widespread implementation. Regulatory Uncertainty



## The Way Forward: Overcoming Challenges and Enhancing Uptake

To address these challenges and encourage greater uptake of the FDA's draft guidance, collaboration and innovation are key. Here are some steps that stakeholders can take:

**Raise Awareness** - Educate stakeholders—including pharmaceutical companies, researchers, and patient advocacy groups—about the value of PROs and the FDA's draft guidance. Highlight success stories where PROs have influenced treatment decisions and improved patient outcomes.

**Standardize Practices** - Develop industry-wide standards for PRO assessments to facilitate consistency and comparability across trials. Collaborative efforts can help validate the FDA's recommendations and promote the use of standardized PRO measures.

**Incorporate PROs Early in Trial Design** - Integrating PRO strategies into trial design from the outset ensures seamless implementation. Oncology trials are complex, and PRO considerations should not be an afterthought. Early planning and consultation with experts can help address potential challenges and improve the quality of PRO data.

**Collaborate and Innovate** - Encourage partnerships between pharmaceutical companies, academic researchers, technology vendors, and patient advocacy groups to advance PRO implementation. Exploring emerging technologies, such as computerized adaptive testing and wearable sensors, can provide novel insights into patient experiences and enhance data collection.

**Embrace Technology** - Leverage digital tools, such as mobile apps and electronic diaries, for PRO data collection. These technologies can enhance patient engagement, improve adherence, and reduce the incidence of missing data, ultimately leading to more reliable and valuable PRO insights.

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## Conclusion

Over the past decade, the inclusion of PRO data in oncology drug labeling has neither been a regulatory requirement nor a widely adopted practice. As a result, much of the valuable data collected on patient experiences during treatment has not been utilized to its full potential. Adopting the FDA's draft guidance provides a framework for sponsors to collect submission-ready PRO data that is more likely to be accepted by the FDA and included in drug labeling.

The FDA's draft guidance represents a significant step toward patient-centered cancer research. By embracing PROs, pharmaceutical companies can enhance their understanding of treatment effects, improve patient outcomes, and provide more comprehensive information to future prescribers and patients. It's time to bridge the gap between guidance and practice, validating these recommendations so that all future oncology trials and patients can benefit.

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