

FDA's guidance on patient reported outcomes provides valuable insight on using PROs to authenticate label claims.

## A Winning PRO Game Plan

Mark Kosinski

**T**he rule book may be new, but the goal is the same: prove to consumers that your product works. One of the keys to success is making sure you use the rule book to your advantage.

The rule book in this case is the FDA's guidance on patient reported outcomes (PROs) issued last December. The guidance provides valuable insight into the FDA's requirements for using PROs to substantiate label claims. As a result, the information in the guidance can be used to facilitate communication with the FDA throughout the product development and clinical trial process. The guidance also standardizes the process for developing PROs and presenting outcomes data so that they'll be acceptable. Ultimately, it puts everyone on the same page.

### What it means

It's true the guidance sets a higher standard for proving the appropriateness of a PRO for a particular application. However, the payoff for meeting that standard is substantial: the opportunity to state claims on labels, inserts, and other promotional materials. Which brings us back to the all-important consumer. The ability to put a product's claims in terms that matter most to consumers, such as the ability to work, have energy, and be happy, improves the chances the product will be accepted and used. It also sharpens the competitive edge by making it easier for the company to:

- Differentiate the product from the competition
- Educate consumers and healthcare providers
- Develop powerful, well-defined marketing communications
- Increase brand awareness

So, what is the FDA looking for exactly? Simply put, they want to know the PRO actually measures what it intends to measure for the patient population in a clinical trial. Among other things, they examine the PRO's characteristics (e.g., the concepts being measured, such as symptoms or effects on a particular function), con-

ceptual framework (e.g., a diagram of the relationships between the questionnaire or items in a PRO and the concepts measured), and content validity (e.g., evidence from qualitative research demonstrating that the PRO measures the concept of interest).

If a PRO is to be used to support a label claim, the FDA suggests determining at the outset of the product development process whether or not a PRO exists that will work. If not, a new PRO can be created from scratch or by modifying an existing PRO. In other words, a PRO game plan is needed, and it is needed early.

### Planning to succeed

QualityMetric Incorporated has developed a comprehensive, step-by-step PRO development process that lays the groundwork for meeting the FDA's requirements. The key stages of our process include:

- **Developing a conceptual framework:** This initial stage involves a literature review, patient and key opinion leader interviews, creation of a disease model, and consultation with governing or regulatory bodies. QualityMetric has access to clinical experts—including physicians representing a broad range of medical specialties—who can assist during this stage.
- **Survey development:** At this stage, we focus on the creation of items or questions for key concepts.
- **Cognitive/pilot testing:** This testing stage allows QualityMetric to gain insight into a respondent's interpretation of items and terms, and identifies items that are vague or difficult to understand.
- **Validation studies:** Validation is a crucial part of the development process in which PRO tools are tested to make sure they measure what is intended. A primary purpose of validation is item reduction—to identify and keep the best performing items.

QualityMetric also provides the documentation the FDA requires to support the use of the PRO. By following this process, we can ensure a scientifically valid PRO that complies with the FDA's guidance.

### A versatile tool

A company's needs should be a driving force behind any PRO game plan. In addition to substantiating label claims, a company most likely has many other needs that a PRO can satisfy. By establishing and following a PRO strategy that spans the entire clinical trial continuum an impressive body of evidence about the benefits of a product and how it impacts the way patients feel and what they are able to do, can be accumulated. The information can be used in many ways:

- Identify profitable new applications and markets for a product by analyzing targeted populations for unmet medical needs.
- Prove the positive impact of a product on patient health and, ultimately, health expenses.
- Answer questions about comparative effectiveness in order to build an economic case for a product with insurers.
- Facilitate understanding of real-world experiences with the product to protect the safety of consumers—and a company's reputation.

PROs can be an important way to ride the wave of consumerism in healthcare. They can become a tool for consumers who don't necessarily understand the clinical jargon that often describes the effectiveness of a product. If they realize—through the use of PROs—that they are more likely to go back to function without limitations, have energy, and be happy, they can make better choices.

Over the past 10 to 15 years, PROs have become an integral component of the clinical trial process. Along the way, healthcare professionals, product manufacturers, and PRO developers have found new and innovative ways to use these tools to design and promote products as well as deliver better care. Whatever the goal, it's important to tap all the resources available to achieve it.

**Mark Kosinski, MA, Vice President and Senior Scientist at QualityMetric, now part of i3, 24 Albion Road, Bldg 400, Lincoln, RI, e-mail: mkosinski@qualitymetric.com.**