Patient-Reported Outcomes in Gastroenterology: Clinical and Research Applications

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Patient-generated reports, also known as Patient-Reported Outcomes (PROs), capture the patients’ illness experience in a structured format and may help bridge the gap between patients and providers. PROs measure any aspect of patient-reported health (e.g., physical, emotional or social symptoms) and can help to direct care and improve clinical outcomes. When clinicians systematically collect patient-reported data in the right place at the right time, PRO measurement can effectively aid in detection and management of conditions, improve satisfaction with care and enhance the patient-provider relationship. This review article summarizes the latest approaches to PRO measuring for clinical trials and clinical practice, with a focus on use of PROs in gastroenterology.


Key Words
Gastroenterology; Patient-reported outcomes; Quality of life

Introduction

Patients typically seek health care because they experience symptoms. Health care providers must elicit, measure and interpret patient symptoms as part of the clinical evaluation. Patient-generated reports, also known as Patient-Reported Outcomes (PROs), capture the patients’ illness experience in a structured format and may help bridge the gap between patients and providers. The United States Food and Drug Administration (FDA) defines a PRO as “any report of the status of a patient’s health condition that comes directly from the patient, without interpretation of the patient’s response by a clinician or anyone else.”

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In addition to their use in clinical practice, PROs also play an important role in clinical trials and other research endeavors. For example, health-related quality of life (HRQOL), a sub-type of
PRO that measures biopsychosocial health, has gained traction as an outcome in clinical research, including clinical trials. HRQOL measures can document patient improvement or decrement over time, and help to estimate the benefits of clinical interventions. In addition, the FDA now considers the patient report in drug approval, and has developed guidance for use of PROs in clinical trials. The National Institute of Health (NIH) has also supported a major PRO initiative, called the Patient Reported Outcome Measurement Information System (PROMIS®; www.nihpromis.org), designed to develop and evaluate several HRQOL domains.10 I will discuss PROMIS in detail later in this article because it is now becoming the new gold standard of PRO measurement. Finally, the rising prominence of the Chronic Care Model, which emphasizes the centrality of the provider-patient relationship in clinical decision making,2,3 places the patient report front-and-center. In short, there is a confluence of scientific, regulatory and political factors that amplify the importance of PRO research.

Gastrointestinal (GI) illnesses can lead to physical, mental and social distress.11 For this reason, patients, providers, investigators and regulators are interested in using PROs to guide clinical decision-making, conduct clinical research and achieve drug approval in GI disorders. Over the last 2 decades, investigators have developed PROs that measure a range of GI symptoms, including physical, emotional and social features of digestive disorders. It is important for GI providers and researchers to be aware of existing PROs, their limitations and how they can be used.

The purpose of this article is to help guide clinicians and investigators by reviewing PRO measurement in gastroenterology. Because much of the work in PROs focuses on functional gastrointestinal disorders (FGIDs), like irritable bowel syndrome (IBS), I will intermittently focus on FGIDs as a model for how to measure and employ PROs for clinical and research purposes.

**Importance of Patient-Reported Outcomes in Clinical Practice**

There are at least four reasons why measuring PROs is relevant in clinical practice. First, any experienced provider knows that traditionally measured biomarkers often fail to correspond with how a patient is actually feeling. For example, in diabetes, clinicians usually measure the hemoglobin A1C level. This value is often used to make treatment decisions, like how aggressively to treat the diabetes, or whether to change to a new medication. But the problem is that some patients may have a low hemoglobin A1C but still feel listless or depressed despite their favorable laboratory values. In contrast, others with unfavorable levels may nonetheless feel upbeat and vigorous. Similarly, an IBS patient with 6 daily bowel movements may share the same problems at work (like difficulty getting the job done because of the constant bother of symptoms) as another IBS patient with 3 daily bowel movements. In both examples the traditional outcome measured by healthcare providers (e.g., hemoglobin A1C levels, bowel movement frequency) fails to capture other aspects of health. In other words, just asking about bowel movement frequency in IBS (for example) is not enough to fully understand how the disease is affecting a patient; it is only part of the picture.

A second reason to measure PROs is that patients rarely value traditional biomarkers in the same manner as providers. For example, patients with hypertension often fail to share the same enthusiasm as their providers in achieving specific blood pressure goals, but are quick to comply with therapy when their hypertension leads to headaches or dizziness. Similarly, some patients with chronic constipation could care less if their therapy allows them to achieve an increase of 1 or 2 more bowel movements per week, but care greatly if the improvement also allows them to eat dinner without worrying about the consequences of their food selection. Measuring HRQOL, in particular, directly acknowledges that patients often value different outcomes than their providers.

Third, PROs provide a key component to understanding the true burden of any disease. Traditional measures of disease burden include the prevalence of a disease, direct and indirect expenditures of a disease, and the worker productivity decrements related to a disease. However, in order to fully appreciate the true burden of a disease, it is also important to appreciate the HRQOL decrement engendered by the disease. The notion of “weighting” diseases not only by their cost and prevalence but also by their HRQOL decrement has an innate sense of fairness and is a fundamental principle of health economics.12 This is important, because it suggests that society and health providers are willing to spend more money to cure diseases that severely impact HRQOL than diseases that only moderately impact HRQOL. For this reason alone it is critical to carefully understand the HRQOL decrement of various diseases, because that information may have policy implication when it comes to developing a healthcare budget.

Fourth, PROs are especially important in diseases that are marked by morbidity, but not mortality. If we only emphasized