Commentary

Clinical Trial Patient-reported Outcomes Data: Going Beyond the Label in Oncology

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ABSTRACT

Purpose: Patient-reported outcome (PRO) data are increasingly being implemented in oncology clinical trial research to evaluate treatment benefit, such as disease-related symptoms, treatment-related adverse events, and health-related quality of life impacts. However, only a small amount of PRO data collected is used to support labeling claims, leaving a substantial amount of data that could be shared by sponsors to further convey treatment benefit from the patient perspective.

Methods: This paper describes how pharmaceutical sponsors can realize the value of PRO data derived from oncology trials with regard to the following stakeholders: payers, health care providers (HCPs), and patient advocacy groups. Further, ideas are presented for integrating PRO data and implementing PRO assessments within oncology, by stakeholder type. Finally, a summary is provided to describe how PRO data can benefit the patient by facilitating better, more symptom-focused care and enhancing treatment decisions.

Findings: With the goal of motivating further use of PRO assessments in oncology, we present examples of how payers utilize PRO data to inform reimbursement decisions (eg, PRO data inform decisions made by Germany's Institute for Quality and Efficiency in Health Care and the United Kingdom's National Institute for Health and Care Excellence); how communication of results with patient advocacy groups can lead to a better understanding of what is important to patients; and how HCPs can use PRO instruments to inform patient treatment decisions through real-world application.

Implications: Integrating PRO data can enhance health care by allowing the patient's voice to carry beyond regulatory decisions and into those made by payers and HCPs, which are crucial to quality care and assessing the value of care. Utilizing PRO assessments and communicating results to key stakeholders in the oncology space can allow sponsors to report treatment benefit and, more importantly, can provide valuable insight into the patient treatment experience. (*Clin Ther.* 2016;38:811–820)
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Key words: Patient-reported outcome, clinical trial endpoints, oncology, reimbursement.

INTRODUCTION

Patient-reported outcome (PRO) measures are increasingly used as end points in oncology clinical trials[1,2] to better understand the patient treatment experience and supplement information afforded by traditional end points. More specifically, PROs can evaluate treatment benefit (such as disease-related symptoms or impacts) directly from the patient perspective.[3] Historically, primary end points in oncology have focused on objective clinical measures, such as survival and tumor reduction. While the importance of these objective measures cannot be debated, PRO instruments can reinforce these end points and provide the patient's

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perspective, documenting how a treatment impacts the way the patient feels (eg, reduces pain), functions (eg, allows them to do more), or copes (eg, manages treatment-related toxicities).

Using PROs to evaluate the efficacy of medical treatments in clinical trials is not new[4–7] and has proven successful in recent years.[8] Regulatory guidance and opinion papers[9–11] have also increased attention to the topic. The US Food and Drug Administration's 2009 PRO Guidance highlights the importance of the patient perspective in drug development and defines a PRO as any "measurement based on a report that comes directly from the patient (ie, study subject) about the status of a patient's health condition without amendment or interpretation of the patient's response by a clinician or anyone else."[9] More recently, a variety of guidances (including those published by the US Food and Drug Administration[12–15] and European Medical Association[10,11]), as well as papers focused on the development and implementation of PRO instruments and interpretation of PRO data, have become available.[16–21] In alignment with these advancements, PRO instruments are being developed, including the PRO version of the Common Terminology Criteria for Adverse Events, an electronic-based system for patient self-reporting of adverse events (AEs) intended to complement the current standard of AE reporting and potentially improve the accuracy and precision of grading AEs in oncology.[22] The PRO version of the Common Terminology Criteria for Adverse Events is intended to provide a method for relaying information directly from patients to clinicians (instead of the traditional method, which entails interpretation through multiple channels, including additional informants and chart review).[22]

PROs are critical to sponsors seeking drug approval by regulators; in addition, some experts have highlighted their value in reimbursement and regimen selection decisions for payers and health care providers (HCPs), respectively.[23,24] Of course, it is the patient that is central to all stakeholders and their decisions (as shown in the Figure) and, therefore, it would benefit stakeholders to understand how to use and interpret PRO data as a means to directly reflect the patient voice. Both understanding and clearly articulating the purpose and context of use for patient-reported questionnaires and data, by stakeholder type, will allow for informed and practical recommendations.

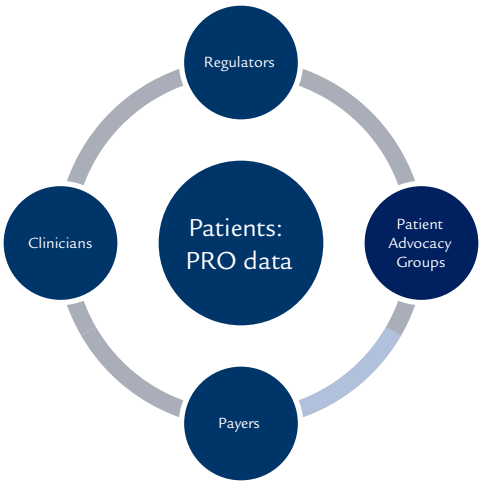

Figure. Patient-reported outcomes data stakeholders.

Much attention has been given to utilizing PRO data to report treatment benefit in regulated trials for product approval and labeling. However, the value of collecting PRO data for purposes beyond labeling and registration (eg, reimbursement and treatment decisions), while certainly contemplated,[19,25] has been less documented. This paper describes how pharmaceutical sponsors can realize the value of PRO data derived from oncology trials in relation to key stakeholders, including ways to communicate these data to payers and HCPs through appropriate and compliant channels. Further, ideas are presented for integrating and implementing PRO assessments within oncology trials, by stakeholder type.

Drug Sponsor: Dissemination of Patient-reported Outcomes Data

Despite their increased use in oncology trials, it is uncommon for a PRO to be incorporated as a primary end point, given the focus on survival and tumor reduction. However, it is increasingly common for PRO measures to be incorporated as secondary or exploratory end points,[1] including measurement of the severity and impact of AEs (PRO version of the Common Terminology Criteria for Adverse Events).[22] While the value of collecting symptoms or toxicities through PRO measures in an oncology trial is significant (as survival might only be important to patients if their symptoms are also reduced), only a

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