The emerging role of patient-reported outcomes (PROs) in FDA hematology and oncology product labels

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Background

The Food and Drug Administration (FDA) defines a patient-reported outcome (PRO) as "any report of the status of a patient’s health condition that comes directly from the patient, without intermediary interpretation of the patient’s response by a clinician or anyone else". In 2009, the FDA published guidance for manufacturers entitled "Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims", which explains how the FDA evaluates PRO instruments used in clinical trials when a manufacturer is seeking a PRO label claim.

In 2013, the Center for Medical Product Development (CDER) published an effectiveness quarterly summary report (QSR) that was developed in response to the Center for Drug Evaluation and Research’s (CDER) need to better understand the extent to which PRO data are being captured in previous clinical cancer research.

Objectives

The main objective of this study was to evaluate the extent to which PRO data are being captured in FDA-approved product labels in hematology and oncology and to identify the reasons why PRO data are not more commonly captured.

Methods

A total of 41 manufacturers were surveyed by email for data regarding label claims from 2006 to 2010. Of these, 18 respondents provided data. The data were evaluated and summarized in Table 1. Data were extracted by two authors (JT and SS) with final approval and review by a third author (AN).

Table 1: Summary of QSR recommendations for incorporation of PROs into CRFs (Basch et al., 2010)

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<th>Objective</th>
<th>Recommendations</th>
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<td>To increase PROs on FDA-approved product labels (summarized in Table 5).</td>
<td>- PRO stakeholders to develop a research agenda to characterize and mitigate the adequacy of existing instruments, the clinically meaningful change, and responsiveness to change. - New questionnaires to be systematically analyzed; the FDA to endorse those suitable to support label claims.</td>
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