Abstract

Donald Patrick, University of Washington, Seattle

Patient-Reported Outcomes (PROs) and the Food and Drug Administration (FDA) Draft Guidance: Key Challenges for Industry

This presentation concerns development and use of patient-reported outcomes (PROs) in clinical trials to evaluate medical products. A PRO is any report coming directly from patients, without interpretation by physicians or others, about how they function or feel in relation to a health condition and its therapy. PRO instruments measure these patient reports, providing a unique perspective, because some effects of a health condition and its therapy are known only to patients. Properly developed and evaluated PRO instruments have the potential to provide more sensitive and specific measurements of the effects of medical therapies, thereby increasing the efficiency of clinical trials that attempt to measure the meaningful treatment benefits of those therapies. Poorly developed and evaluated instruments may provide misleading conclusions or data that cannot be used to support product labeling claims. Major challenges arise from FDA's perspective in using PRO instruments, measures, and endpoints to support treatment benefit claims in product labeling. These challenges highlight the need for sponsors to formulate desired labeling claim(s) prospectively, to acquire and document information needed to support these claim(s), and to identify existing instruments or develop new and more appropriate PRO instruments for evaluating treatment benefit in the defined population in which they will seek claims.