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Consistent Patient-Reported Outcomes

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Health-related quality of life (HRQL) measures extend patient outcome assessment beyond survival, adverse effects, and clinical efficacy, and reflect the patient's perspective on the impact of disease and its treatment on functioning and well-being. An important objective for evaluating HRQL outcomes is to demonstrate the value of new pharmaceutical treatments relative to other competing treatments. The pharmaceutical industry's intent in supporting clinical trials with HRQL measures is to achieve labeling and/or promotional claims for marketing to physicians, pharmacists, health-care decision makers, and patients [1,2].

Morris and Miller [3] provide an informative and useful policy analysis of the evidence required for HRQL and other patient-reported outcome (PRO) claims for labeling or promotion. They argue that there is apparent inconsistency in the standards of evidence used that depends on whether the HRQL findings appear in the product label, and where on the label they appear. The result is that there is evidence that promotional claims for HRQL and other PRO effects require higher standards of evidence if no HRQL information is included in the product label. These authors further recommend that the level of substantiating evidence for an HRQL or PRO claim for advertising should be based on where in the product label this information would be placed. In most cases, HRQL findings would be included in the Clinical Studies section of the label and would therefore require evidence from at least one well-controlled and scientifically adequate clinical trial.

Over the past 10 years, there has been some inconsistency within the FDA, leading to confusion among HRQL researchers, industry personnel, and clinicians in terms of the standards required to support promotional or labeling claims of HRQL effects. Clearly, a regulatory guideline for the placement of HRQL and PRO findings in product labels and the expected substantiating evidence for HRQL and PRO claims would assist FDA reviewers and the pharmaceutical industry. It is unlikely that the FDA will issue guidance on HRQL labeling and promotional claims in the near future. However, FDA reviewers and advisory committees are evolving in their understanding of HRQL and patient outcome assessment, and there is an increasing level of consistency in the FDA's decision making regarding HRQL promotional and labeling claims. From the perspective of the FDA, the HRQL and other PROs assessed in pivotal clinical trials and new drug applications add to the understanding of the effectiveness of new treatments. Consequently, PROs are treated as clinical efficacy end points, and substantial evidence is needed to support an HRQL labeling or promotional claim.

There is a need for consistent standards of evidence to support labeling and/or promotional claims of HRQL benefits associated with a pharmaceutical product. This evidence needs to be based on state-of-the-art HRQL research, but what amount of evidence is sufficient for labeling vs. promotional claims? Are the results from one adequate and well-controlled clinical trial sufficient for substantiating an HRQL or PRO claim for advertising purposes? The article by Morris and Miller [3] provides an informative starting point for discussing relevant evidentiary standards, whether based on the FDA's current standards or the Federal Trade Commission's (FTC) competent and reliable standards, for evaluation of HRQL and PRO claims.

Statements pertaining to HRQL effects must be supported by well-controlled clinical trials, psychometrically sound instruments, and adequate statistical methods [1,2]. In many cases, a single clinical trial with an adequate research design, sufficient sample size and power, attention to HRQL instrument selection and data collection procedures, and appropriate statistical analysis would be sufficient to provide useful information on HRQL outcomes.

The challenge for both industry researchers and regulatory agency reviewers is to reach a consensus on the standards for scientific evidence required to support HRQL and PRO claims for labeling and advertising. Regulatory agencies need consistent standards for labeling and advertising claims. These standards must be based on consensus within the

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HRQL scientific community in terms of state-of-the-art research methods, instrumentation and assessment procedures, and the flexibility to accommodate new measurement research and methodologies. The communication of patient-reported health outcomes must be clear, unambiguous, and based on adequate scientific methods. In this way, physicians, patients, and health-care decision makers will have confidence in information about the impact of treatment on patient functioning and well being.

References

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