Developing Pediatric Patient-Reported Outcome Instruments for Clinical Trials to Support Medical Product Labeling—An ISPOR Task Force Publication

Louis Matza, PhD, Senior Research Scientist, Outcomes Research

As the chair of the ISPOR task force focusing on patient-reported outcomes in children and adolescents, I am proud to announce the publication of a new PRO Good Research Practices Task Force Report in *Value in Health* titled "Developing Pediatric Patient-Reported Outcome Instruments for Clinical Trials to Support Medical Product Labeling." This report provides recommendations that will assist researchers who develop and use patient-reported outcome (PRO) instruments in child and adolescent samples. The good research practices described in this report will help ensure that children's experience of disease and treatment are accurately measured in clinical trials, potentially leading to more effective assessment of treatment outcomes in pediatric samples.

The recommendations are based on the consensus of an interdisciplinary group of researchers who were assembled for a task force associated with the International Society for Pharmacoeconomics and Outcomes Research (ISPOR). In addition to health outcomes researchers from academia, research organizations, and industry in the United States and Europe, the task force included a pediatrician from the Division of Anti-Infective Products at the U.S. Food and Drug Administration (FDA). All task force members were chosen based on their experience in PRO assessment and research focused specifically on children and adolescents.

Recommendations were specifically targeted toward development and use of PRO measures that may be used to support medical product labeling and inform regulatory decisions.

The authors discuss five good research practices, while discussing issues of great interest to researchers and professionals who use and develop PRO instruments for assessment of children and adolescents. The task force recommendations are supported by a broad review of health outcomes research in children and adolescents, and numerous citations are provided.

The good research practices discussed in the report include:

1. **Consider developmental differences and determine age-based criteria for PRO administration.** Four age groups are discussed based on previous research (<5 years old, 5 to 7, 8 to 11, 12 to 18).
2. **Establish content validity of pediatric PRO instruments.** This section discusses the advantages of using children as content experts, as well as strategies for qualitative research with children.
3. **Determine whether an informant-reported outcome instrument is necessary.** The distinction between two types of informant-reported measures (proxy vs. observational) is discussed, and recommendations are provided.
4. **Ensure that the instrument is designed and formatted appropriately for the target age group.** Factors to consider include health-related vocabulary, reading level, response scales, recall period, length of instrument, pictorial representations, formatting details, administration approaches, and electronic data collection (ePRO).
5. **Consider cross-cultural issues.**

In areas where supporting evidence is limited or where general principles may not apply to every situation, this task force report identifies factors to consider when making decisions about the design and use of pediatric PRO instruments. The report also suggests directions for future research needed to further develop this important area of research.

The full citation for the task force report is: Matza LS, Patrick DL, Riley AW, Alexander JJ, Rajmil L, Pleil AM, Bullinger M. Pediatric Patient Reported Outcome Instruments for Research to Support Medical Product Labeling: Report of the ISPOR PRO Good Research Practices for the Assessment of Children and Adolescents Task Force. *Value in Health.* Jun 2013; 16(4):461–479. The report can be accessed on the ISPOR website at: http://www.ispor.org/workpaper/PROchildrenadolescents/Matza_et_al_2013_ISPOR_Task_Force_PROs_in_Children.pdf.

For more information, please contact Louis.Matza@evidera.com.