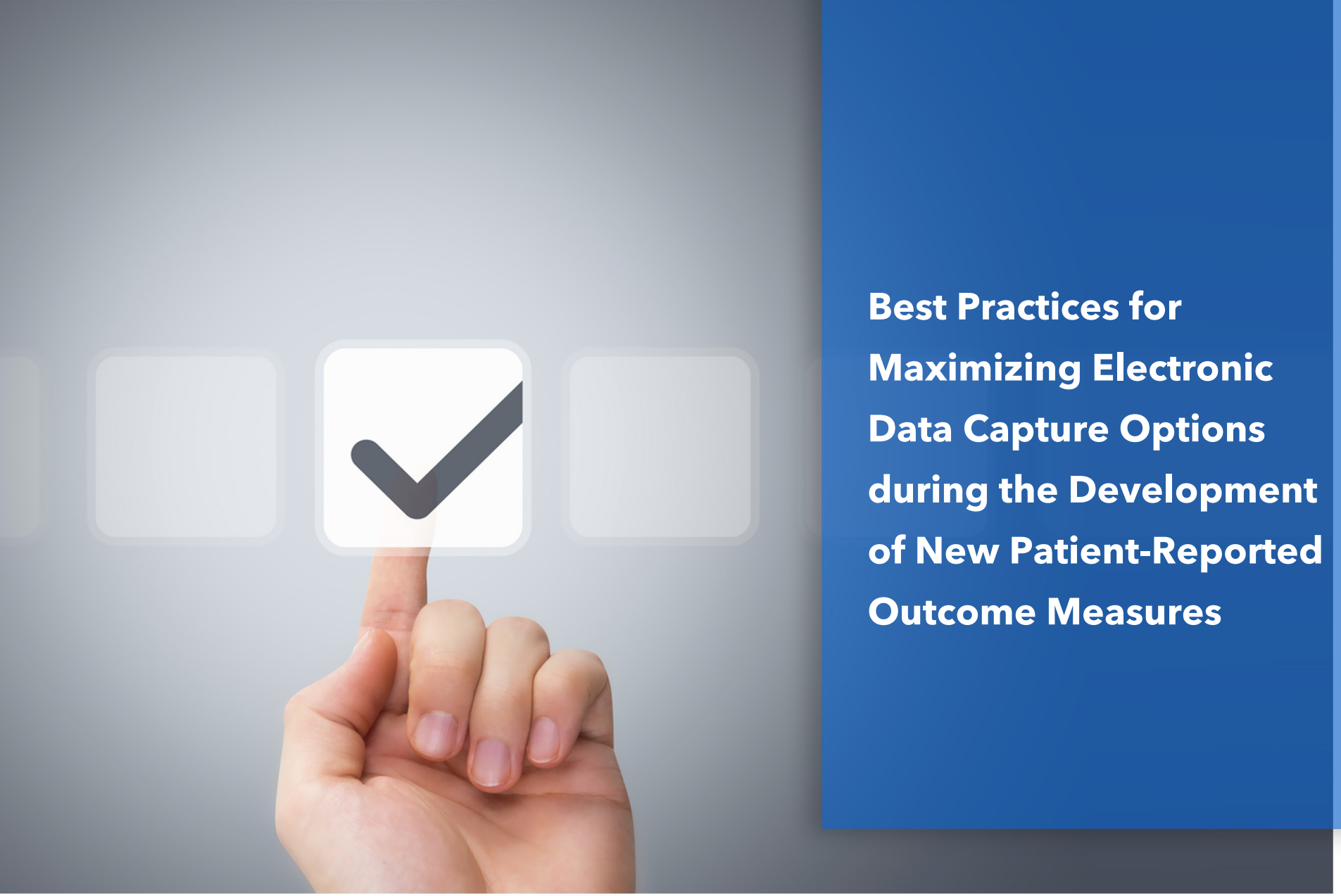

Best Practices for Maximizing Electronic Data Capture Options during the Development of New Patient-Reported Outcome Measures

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c-path.org/programs/epro

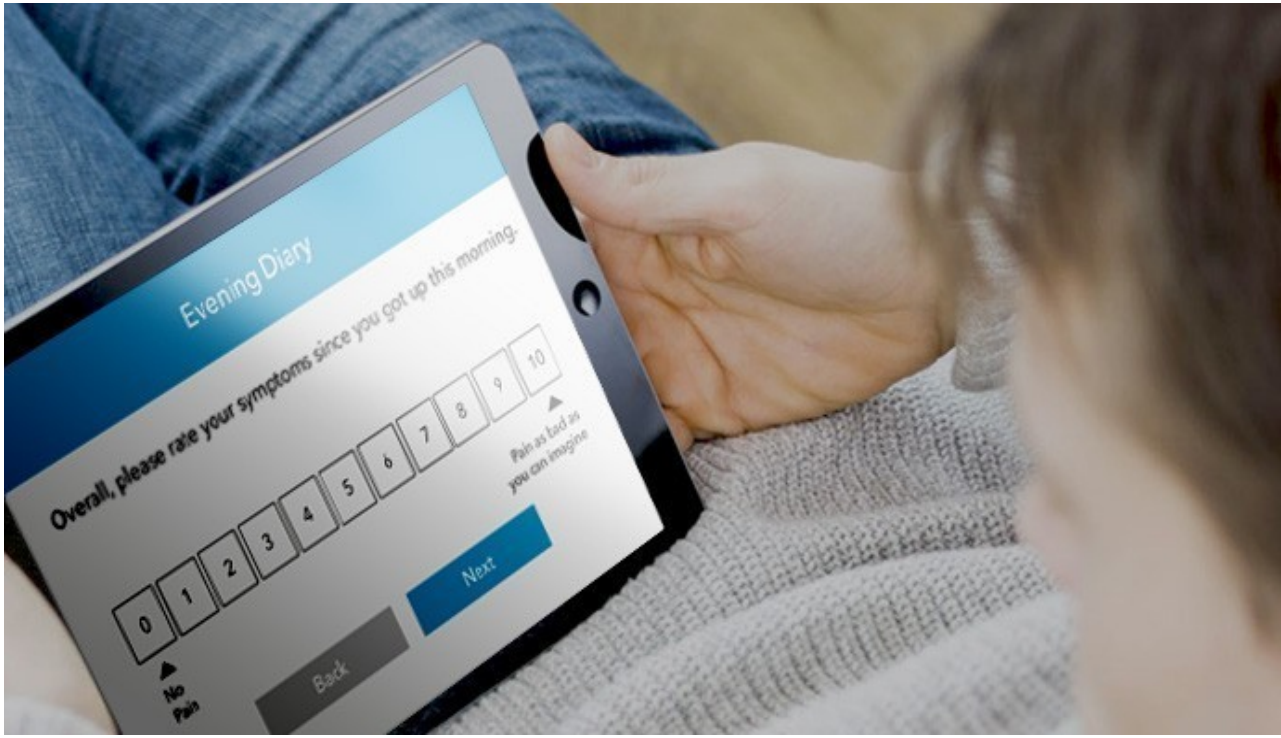
Scope

This document addresses issues that should be considered when developing patient-reported outcome (PRO) measures to enable their implementation on any available data collection mode.

The assumptions and nomenclature related to the application of this document to a new measure development project are listed below.

Assumptions

- This document is intended for use by those embarking on de novo PRO measure development.
- The considerations below are independent of the mode used for data collection.
- This document does not replace or supersede guidance for the development of PRO measures for use in assessing endpoints in clinical trials or the translation and cultural adaptation of PRO measures. Selected foundational references are provided below.



References for Content Validity:

- US Food and Drug Administration. Guidance for Industry: Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims, December 2009. Available at: http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM193282.pdf.

- Patrick DL, Burke LB, Gwaltney CJ et al. Content validity - Establishing and reporting the evidence in newly-developed patient-reported outcomes (PRO) instruments for medical product evaluation: ISPOR PRO good research practices task force report: Part 1 - Eliciting concepts for a new PRO instrument. *Value in Health* 2011;14:967-977.

- Patrick DL, Burke LB, Gwaltney CJ, et al. Content validity - Establishing and reporting the evidence in newly-developed patient-reported outcomes (PRO) instruments for medical product evaluation: ISPOR PRO good research practices task force report: Part 2 – Assessing respondent understanding. *Value in Health* 2011;14:978-988.

References for Translation and Cultural Adaptation:

- Wild D, Grove A, Martin M, et al. Principles of good practice for the translation and cultural adaptation process for patient-reported outcomes (PRO) measures: Report of the ISPOR task force for translation and cultural adaptation. *Value in Health* 2005;8:95-104.

- Wild D, Eremenco S, Mear I, et al. Multinational trials – Recommendations on the translations required, approaches to using the same language in different countries, and the approaches to support pooling the data: The ISPOR patient-reported outcomes translation and linguistic validation good research practices task force report. *Value in Health* 2009;12:430-440.

- Brislin RW. The wording and translation of research instruments. In: Lonner WJ, Berry JW, eds. Field methods in cross-cultural research. Beverly Hills: Sage, 1986:137-164.

Nomenclature

Throughout this document certain terms will be used synonymously. For example, when referring to a PRO measure, the terms *instrument, tool, questionnaire,* and *scale* may be used interchangeably.

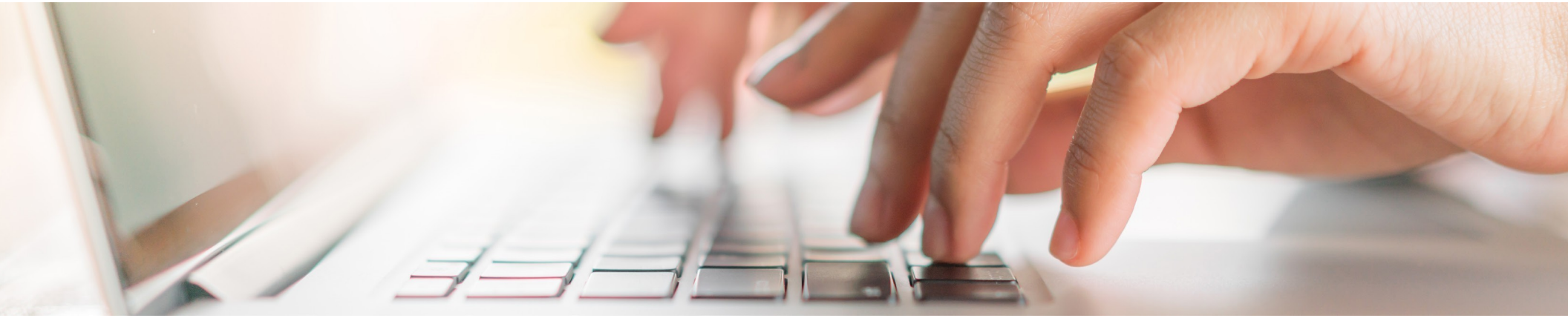
The use of the words *mode* and *method* in this document differs from that used in the FDA's PRO Guidance. The PRO Guidance defines *modes of administration* as self-administration, interview, or a combination of both. Further, *data collection methods* are defined to include paper-based, computer-assisted, and telephone-based assessments. However, an informal review of the articles included in a meta-analysis conducted by Gwaltney et al (2008) suggests a different use of this terminology in the ePRO literature. For the purposes of this document, the term *data collection mode* refers to various platforms available for instrument administration (i.e., paper and electronic platforms).

General Considerations

- Consider characteristics of the target population and therapeutic area for which the instrument is intended for use.
  - Consider functional abilities associated with the target population (e.g., diabetes-related vision problems, Ménière's disease-related hearing loss, Parkinson's disease-related tremors, stroke-related physical or cognitive impairment).
  - Consider how frequently the concepts to be measured occur or change. The recall period and administration frequency should reflect this consideration.
- Anticipate that the instrument will be translated into other languages.
  - The cultural appropriateness of the instrument's items and responses should be considered (e.g., avoid idiomatic expressions).
  - Translatability assessment should be conducted early in instrument development See Acquadro C, Patrick DL, Eremenco S, Martin ML, Kuliś D, Correia H, Conway K. Emerging good practices for Translatability Assessment (TA) of Patient-Reported Outcome (PRO) measures. Journal of Patient-Reported Outcomes 2018;2:8.
  - Translated text is likely to be longer than US English text which will impact formatting in small-screen devices
- Consider subject burden with respect to the length of the instrument, time needed for completion, and cognitive complexity.
  - Consider whether other PRO tools are likely to be administered with the new PRO instrument.

- In addition to the characteristics of the instrument, it is also important to consider additional factors that could influence the appropriateness of migration to each respective mode.
  - Instrument developers should provide recommendations about the appropriate data collection modes via websites or user manuals.
- Consider regions of the world where the instrument will be utilized.
  - Consider infrastructure for collection of data electronically (e.g., internet connectivity variation).
  - Assume that translated text will take more space than US English so keep the content compact/brief where possible.

    There are direct implications of wording/phrasing changes for how the instrument is formatted on the data collection mode.
- Consider whether branching or skip logic, real-time edit checks, calculations, and reminders will be incorporated into the measure.
  - Make explicit recommendations for administration window (the time period during which the measure is made available for completion in electronic modes)



Instructions

- Use mode-neutral language in instructions where possible. There are often key words and phrases within instructions that pertain to specific modes (including paper). Mode-neutral language includes words and phrases that can be used and understood on various data collection modes.
- Instructions should be clear and succinct.
- If there is a time frame for recall, be sure that it appears or is heard with every item for which it is relevant, not just once at the beginning of a series of items.

- Examples of mode-specific language:
  "Using the pen/pencil, circle the response …,"
  "Using the numbers on your phone, press the number of the response…,"
  "Using the mouse, click on the response…."
- Examples of mode-neutral language:
  "Select the response…,"
  "Choose the response…,"
  "Enter the response…."

Item Stems

Item stems are generally incomplete statements or direct questions. If the same incomplete statement is used for multiple items, each item should be self-contained (i.e., include the full stem and response options, avoid split stems). Instructional language should be left out of item stems where possible and appropriate.

- Example of split stem (to be avoided):
  During the past 4 weeks, how much has your pain interfered with:
  1. Vigorous activities such as running or heavy lifting?
  2. Moderate activities such as climbing a flight of stairs?
- Example of complete items:
  1. During the past 4 weeks, how much has your pain interfered with vigorous activities such as running or heavy lifting?
  2. During the past 4 weeks, how much has your pain interfered with moderate activities such as climbing a flight of stairs?

Response Sets/Scales

- The use of discrete response sets (e.g., yes/no, true/false) should be limited to constructs possessing nominal or ordinal level data.
- Continuous response scales (e.g., numeric rating scale) are appropriate for use with ordinal, interval, and ratio level data.
- Consider the feasibility of implementing the response set/scale across data collection modes.
  - Different technologies may offer the use of response aides (e.g., spinner/counter) that cannot be operationalized on all platforms.

Considerations for Usability Testing

- Test instruments with subjects from the intended target population with the intended level of training and supervision (e.g., test field-based and site-based PRO instruments with patients and ClinRO instruments with clinicians and patients, if appropriate).
- Use the actual mode on which the instrument will be deployed for usability testing, not screenshots (e.g., if the instrument will be deployed on a tablet, use a tablet and not a handheld device for testing).