Best Practices for Migrating Existing Patient-Reported Outcome Measures to a New Data Collection Mode

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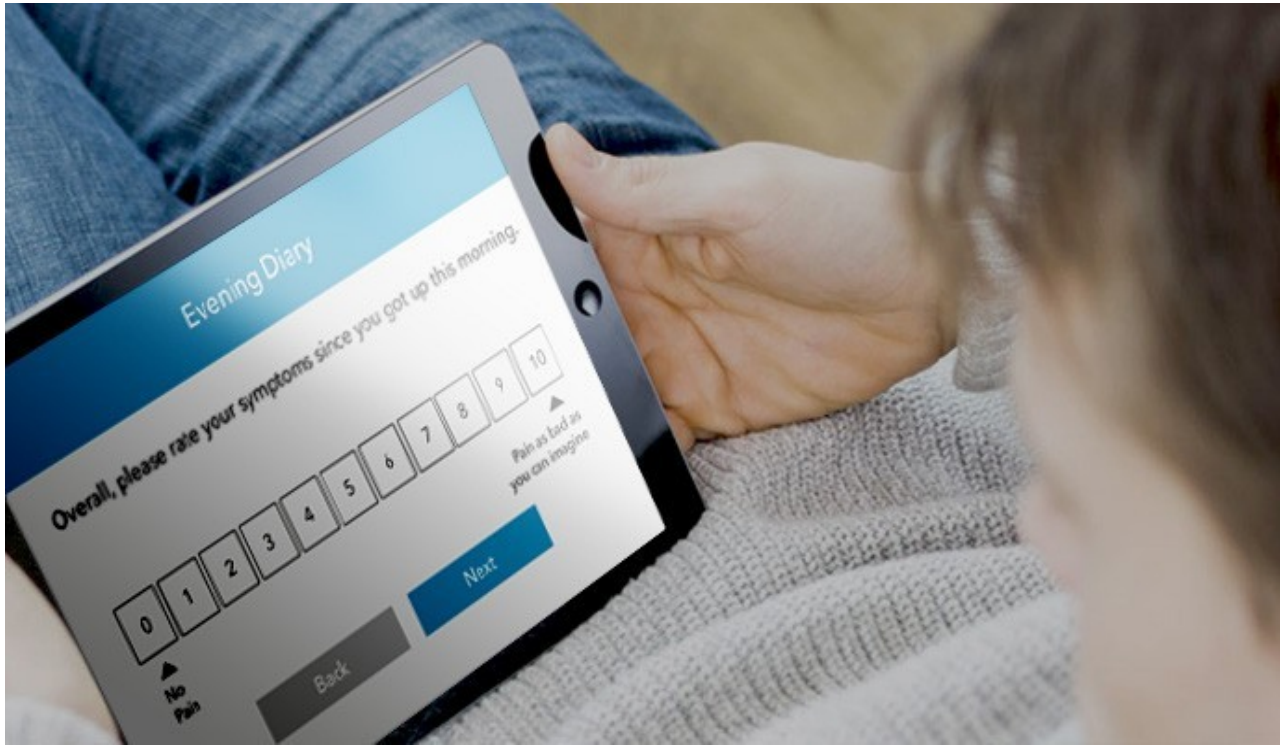
Scope

This document addresses issues that should be considered when migrating an existing patient-reported outcome (PRO) measure from its current data collection mode to any other available data collection mode (e.g., paper, interactive voice response [IVR] system, tablet, Web, handheld).

The assumptions and nomenclature related to the application of this document to the migration of an existing measure are listed below.

Assumptions

- This document is intended for those embarking on the migration of an existing measure to a new mode of data collection from its original mode.
- 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 Migration:

- Eremenco S, Coons SJ, Paty J, Coyne K, Bennett AV, McEntegart D, ISPOR PRO Mixed Modes Task Force. PRO data collection in clinical trials using mixed modes: report of the ISPOR PRO mixed modes good research practices task force. *Value in Health* 2014, 17:501-16.

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 PRO measure administration (i.e., paper and electronic platforms).

General Considerations

Context of Use

- Consider characteristics of the relevant patient population and therapeutic area (i.e., context of use) for which the measure is intended for use.
  - Consider functional abilities or limitations 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 impairmen
  - Examples:
    - Larger fonts should be used when the measure is used by patients with visual impairments.
    - The use of a tablet could be considered rather than a smartphone when dealing with patients with hand tremors
- Trial planning should consider infrastructure for collection of data electronically (e.g., internet connectivity, cellular/mobile service).

Language Considerations

- Consider regions of the world where the measure will be utilized
- Assume that translated text will likely take more space than US English
- There are direct implications of wording/phrasing changes for how the existing measure is formatted in a new mode
- Certain formatting does not translate well (e.g., some fonts, capitalization, using all capitals for emphasis, and underlining)

Measure Characteristics

- Consider the setting (e.g. respondent's home, study site) in which the instrument will be completed.
  - What are the restrictions/considerations for each setting?
  - Examples:
    - Best device for the data collection setting (field vs. site-based)
    - Data transmission capabilities
    - Ease of connectivity to a help desk for support
- Consider if multiple modes of the measure will be used together (i.e., mixed modes within a study) and whether it will be necessary to demonstrate equivalence between modes.

- Consider subject burden and the length of the measure, as well as the overall length of the battery of assessments being deployed.
  - Consider the subject burden of completing the measure (e.g., the amount of time to complete the measure, the frequency of completion, cognitive burden)
  - Progress bars or a menu of the measures to be completed could be used to show study subjects their progress through the questionnaire when using a lengthy measure or multiple measures.
- Consult the measure developer about the available modes of administration
- Consider the characteristics of the measure and the appropriateness of migration to each respective mode.
  - Use terminology and wording that are as close as possible to the original measure.
  - Length of measure: number of items
  - Length of item text: words per item
  - Length and structure of response options
    - Complexity (15 responses to evaluate degree of symptom change versus a simple “Yes” or “No”)
    - Visual analog scale (VAS)
    - Numeric rating scale (NRS)
    - Verbal rating scale (VRS)
    - Visual elements (e.g., body diagram)

Electronic Considerations

Electronic technology provides many potential data collection benefits that do not exist at all on paper, such as seamless skip logic, real-time edit checks, calculations, and alarms. The impact and benefits of these should be considered and evaluated as part of the migration; avoid focusing solely on what exists if the original version of the PRO measure is on paper.

- Consider the tradeoff between the benefits of migrating onto an electronic data collection mode versus the potential impact on the original psychometric properties of the questionnaire
- Depending on the level of change, additional testing may be required
- See: Coons SJ, Gwaltney CJ, Hays RD, et al. Recommendations on evidence needed to support measurement equivalence between electronic and paper-based patient-reported outcome (PRO) measures: ISPOR ePRO good research practices task force report. *Value in Health* 2009;12:419-429.

Instructions

- Modification may be necessary for the instructions to make sense in the context of the target mode.
- Use platform-neutral language in instructions where possible.
- Instructions should be clear and succinct.

- Instructions need to be appropriate to the actions of the target mode. For example ‘circle the one answer…’ may become ‘choose the one answer…’

Items

- Items, including full item stem and all response options, should be implemented as a single item per screen.
- 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 to avoid split-stems).
- Example of split item 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 item stem:

  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?

- Instructional language should be left out of item stems where possible and appropriate.
  - If there is a time frame for recall, be sure that is presented with every item for which it is relevant, not just once in the instructions, or at the beginning of a series of items.
- If instructional language is included in the item, then mode-neutral language should be used where possible.
- When migrating to an electronic mode that requires visual processing of the items, full item stem text and all response options should be visible on the screen at all times.
  - Although not recommended, if the full item stem and all response options cannot be displayed on the screen, possible workarounds include toggling, scrolling, hover notes, and split screens.

Response Scales

- Consider the nature of each response scale in the measure to evaluate the appropriateness for migration to the target mode.
- The measure should require the subject to enter an active response to each item. If a respondent does not complete an item, the data should be recorded as missing or no response.
- Length of response options and number of response options may have an impact on appropriateness for migrating to certain modes.
- It is important to consider how edit checks (e.g., the respondent is alerted to re-enter his or her response if an out-of-range value was entered or the response is missing) would be implemented for alternative modes. If possible, it is advisable to keep the edit checks consistent across modalities.
- Item branching logic is an important factor to consider for the migration to electronic modes.
- Verbal anchors for visual analog scales or numeric rating scales may be difficult to place at the end of the response scale on small screen-based devices. Consider using an upright mark to link the extreme value with the anchor text as shown:

- See O'Donohoe P, Lundy JJ, Gnanasakthy A et al. Considerations for Requiring Subjects to Provide a Response to Electronic Patient-Reported Outcome Instruments. *Therapeutic Innovation & Regulatory Science* 2015;49:792-796.

- For example, handheld devices have limited screen space/area and thus careful consideration needs to be made about the appropriateness of implementing long item stems and/or response options on these data collection modes