Content Validation of the Most Bothersome Symptom – Essential Tremor Questionnaire (MBS-ET): A Cognitive Debriefing Study

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Objective

To evaluate the content validity of the Most Bothersome Symptom – Essential Tremor Questionnaire (MBS-ET) in US-based adults with essential tremor (ET).

Background

ET affects 1% of the general population and 4-5% of individuals over age 65[1] and significantly impacts activities of daily living (ADLs) and quality of life.[1, 2]

Patient-reported outcome (PRO) instruments bring the patient perspective into the drug-development process. Personalised PRO instruments can be used in clinical research to examine treatment benefit in relation to what is most relevant to each patient at baseline.[3]

To determine suitability for use, PRO instruments supporting clinical trial endpoints should be content-validated in the target population.[4-7]

The MBS-ET is a novel, personalised PRO instrument developed to assess the symptoms and functional impairments related to tremor that patients find most bothersome.

Methods

Cognitive debriefing interviews were conducted via video-conference with adults with ET across three iterative rounds. Ethical approval was obtained prior to data collection. All participants provided informed consent.

Potential participants were identified by a specialist recruitment agency and were screened against the study inclusion/exclusion criteria.

Structured interviews were conducted following an interview guide; a "think-aloud" technique was utilised to explore patient comprehension and relevance of MBS-ET content. Perceived conceptual comprehensiveness and responder burden (i.e., length) were also assessed.

Transcripts were analysed using content analysis.[8] Data were reported using participant ID codes.

Evidence-based revisions were made to the MBS-ET between rounds, until no further modifications were required.

Further detail on IRB approval, inclusion/exclusion criteria, participant ID coding, and the interview process, and an overview of MBS-ET V1.0 content is provided in the supplementary materials.

Results

N=10 participants were recruited across rounds (50% male; mean age: 60.4 years; mean years since diagnosis: 6.03), including individuals with self-reported mild to severe ADL impacts.

Figures 1 and 2 provide an overview of participant comprehension of items and the perceived relevance of content in Round 3.

The MBS-ET was conceptually comprehensive; no concepts were consistently reported as missing. Additionally, the MBS-ET includes an open-text response option for "other" symptoms/ADL impacts and to rate level of bother on these concepts.

MBS-ET response options and the recall period ("7 days") were generally well understood. All participants asked considered the length to be acceptable.

All MBS-ET concepts were considered patient-relevant, with items assessing upper-limb tremor most frequently endorsed. Across rounds, revisions were minor and by Round 3, content was well understood, and no further revisions were required.

Demographic and clinical characteristics, further detail on participant feedback, item-tracking matrices and an overview of participant relevance and comprehension across all rounds are provided in the supplementary materials.

Figure 1 Patient understanding and relevance of the MBS-ET symptom module (Round 3)

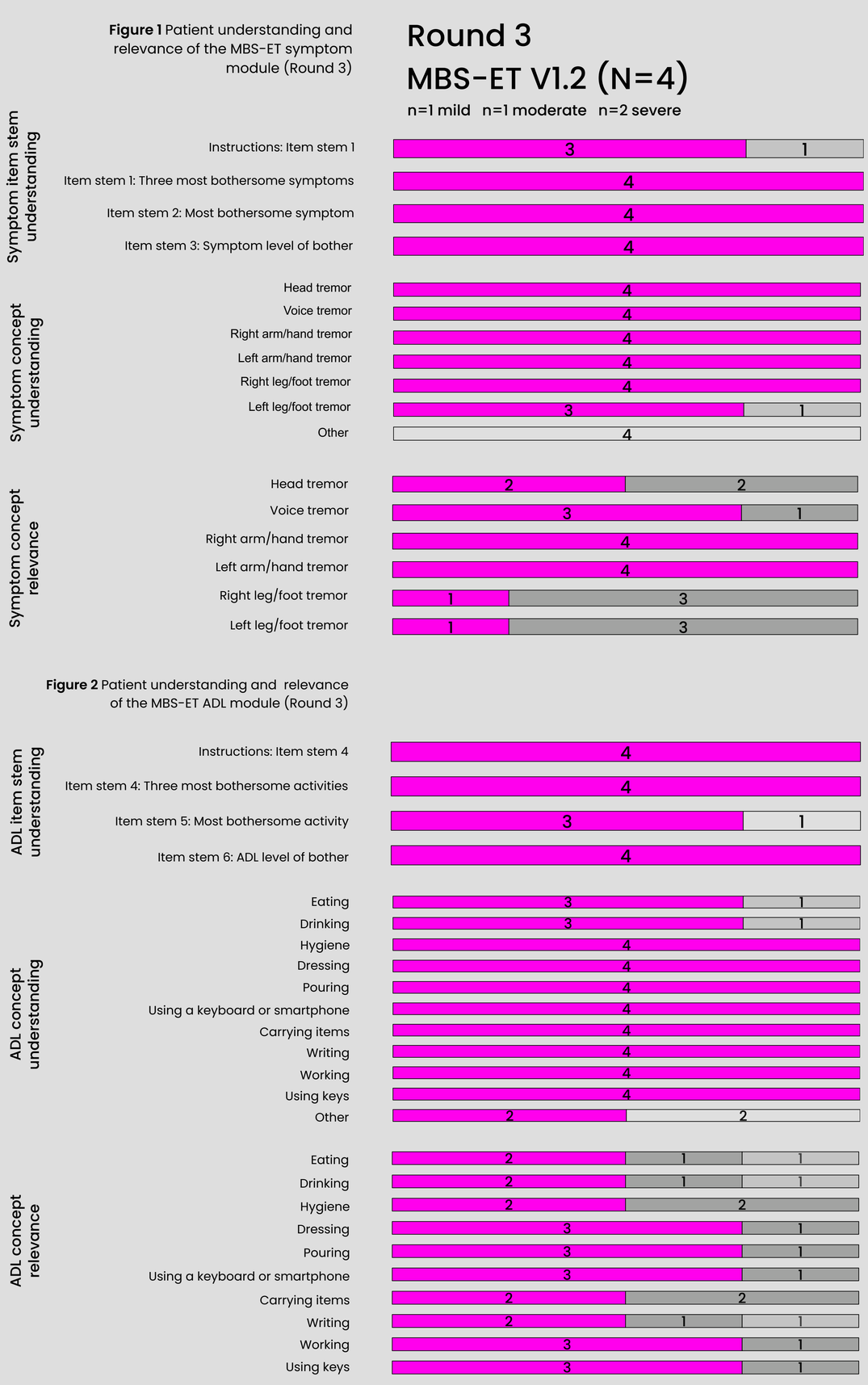

Figure 2 Patient understanding and relevance of the MBS-ET ADL module (Round 3)

Illustrative quotes – patient comprehension and relevance

Example: *Please select up to 3 symptoms that have bothered you the most in the past 7 days. Right arm/hand tremor*

> *"Um, that is when your hand um .. or, or your arm, um, shakes... Slight — it's like, you know, it moves slightly without being provoked."*
>
> P019-MOD – demonstrates symptom understanding

> *"...my hands, it seems like they're shaking, um, I- it's hard for me to hold things still, okay? Um, like I, I don't, I won't pour anything out of a pitcher or a glass, um ... I can use both hands but that's even, that's not even smart to do."*
>
> P020-MOD, demonstrates symptom relevance

> *"...it's asking me to select up to three symptoms that have bothered you in the past seven days, so yeah, like designate er, which symptom applies to me."*
>
> P018-MOD – demonstrates item stem understanding

Conclusions

1. After minor revisions, the MBS-ET V2.0 demonstrated strong content validity in adults with ET. All item stems, symptom/ADL concepts and the 'other' option were well understood, and content was determined to be relevant.
2. When used in clinical research, the MBS-ET may help facilitate consideration of the individual patient perspective into the development of novel ET treatments.
3. The psychometric validity of the MBS-ET V2.0 should be investigated prior to use in clinical studies.

Limitations: a small sample size was utilized in the current study, which may limit the generalizability of findings. Nonetheless, a variety of severity levels (tremor-related impact on ADL) was represented in the sample and sufficient evidence was obtained to inform all revisions and evaluate content validity.

Scan for full details in supplementary materials

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