Content Validation of the Patient Attainment Scale – Essential Tremor (PAS-ET)

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Objective

To evaluate the content validity of the Patient Attainment Scale-Essential Tremor (PAS-ET) in US-based adults with essential tremor (ET).

Background

ET is one of the most common movement disorders[1] and can significantly impact ability to perform activities of daily living (ADLs).[2]

Patient-reported outcome (PRO) measures are essential for evaluating treatment benefit from the patient perspective. The FDA has recently recognised the value of personalised outcome measures, such as Goal Attainment Scales (GAS), in clinical research.[3] GAS measures typically require both patient and clinician input to formulate and evaluate goals, and goal setting in a placebo-controlled setting may not be appropriate.[3,4]

The PAS-ET is a novel, personalised, PRO measure of tremor-related impacts on ADLs. It was developed to assess the importance, current functional ability and level of improvement considered meaningful for each item.

The PAS-ET is less resource-demanding than GAS measures as no clinician involvement is required and addresses potential limitations of GAS measures in setting goals that are not achievable within the applicable patient population or trial setting (e.g. goal setting when in placebo group).[3]

To determine suitability for use, PRO measures supporting clinical trial endpoints should be content-validated in the target population.[5-8]

Methods

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

Potential participants were identified by a specialist recruitment agency and were screened against the study inclusion/exclusion criteria. Recruitment targets were used for age and severity of tremor-related ADL impacts.

Interviews followed a structured guide and employed a “think-aloud" technique to explore patient-comprehension and -relevance of PAS-ET content. Perceived conceptual comprehensiveness and responder burden (i.e., length) were also assessed.

Transcripts were analysed using content analysis.[9] Data were reported using participant ID codes.

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

Further details on IRB approval, inclusion/exclusion criteria, recruitment targets, participant ID coding, and the interview process, and an overview of PAS-ET V1.0 content are provided in the supplementary materials.

Results

A total of N=22 participants were recruited (mean age: 64.2 years; 59% male; mean years since diagnosis: 8.7) across rounds, including individuals with self-reported mild to severe ADL impacts (Figure 1).

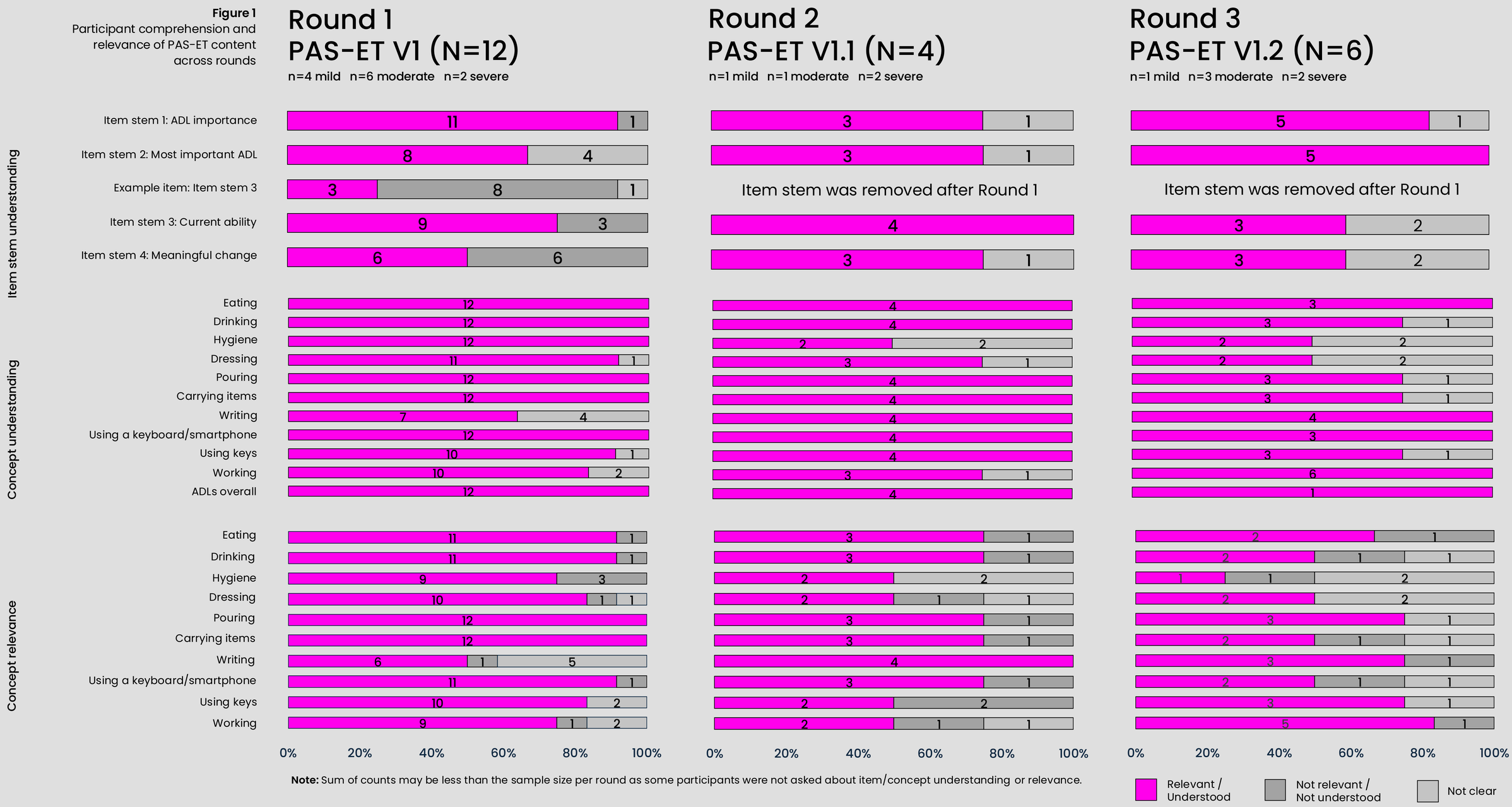
Figure 1 provides an overview of participant comprehension and the perceived relevance of content across rounds.

No ADLs were consistently reported as missing from the PAS-ET and most participants indicated it was an acceptable length. Response options and the recall period (“current”) were generally well understood.

Revisions to the PAS-ET made following Round 1 included the removal of an example item and the wording of two items. Updates following Round 2 were minor. By Round 3, content was well understood, and no further revisions were required.

Demographic and clinical characteristics, further details on participant feedback and item tracking matrices are provided in the supplementary materials.

Figure 1
Participant comprehension and relevance of PAS-ET content across rounds



Note: Sum of counts may be less than the sample size per round as some participants were not asked about item/concept understanding or relevance.

Illustrative quotes – patient comprehension and relevance

Example: *How important is it that a treatment for essential tremor improves your ability to perform the following activity of daily living? Eating*

> ***“It’s asking me if there was a treatment for essential tremors, if it improved how I eat, how important would that be?”***
>
> P005-SEV – demonstrates item stem understanding

> ***“Sometimes when I’m eating, my hand trembles getting the fork to my mouth.”***
>
> P006-MLD – demonstrates ADL understanding and relevance

Scan for full details in supplementary materials

Conclusions

1. The revised version of the PAS-ET (V2.0) demonstrated good evidence of content validity.
2. When used in clinical research, the PAS-ET allows researchers to consider patients’ individual priorities and definitions of meaningful improvement when examining the benefit of treatments on tremor-related ADL impacts. When utilised, the PAS-ET may help facilitate a patient-centric approach to the clinical development of novel treatments.
3. Prior to use in clinical studies, the psychometric validity of the PAS-ET V2.0 should be investigated.

Limitations: All Round 3 participants only partially debriefed the PAS-ET due to time constraints in the interview. However, sufficient evidence was obtained to inform all revisions and evaluate content validity.

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