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Establishing content validity of patient-reported outcome (PRO) measures in extrapulmonary neuroendocrine carcinomas (epNEC): A cognitive debriefing (CD) interview study

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BACKGROUND

Background: Extrapulmonary neuroendocrine carcinoma (epNEC) is a rare, aggressive, high-grade neuroendocrine cancer arising outside the lungs, with heterogeneous symptoms based on the location of the tumors. Utilizing patient reported outcome (PRO) measures in clinical trials is key for capturing the patient perspective, ensuring that their symptoms and daily life experiences are well reflected in evaluating treatments. This study assessed the content validity of PROs including relevance, comprehension, and recall periods of selected PROs for use in patients with epNEC.

METHODS

Methods: Following the *ISPOR PRO Good Research Practices* guidance and given the rarity of epNEC, a 3-step systematic approach was used. First, three oncologists with epNEC expertise assessed relevance of the PRO version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE) selected items. Next, a focus group of four patient representatives and two additional oncologists assessed the European Organisation for Research and Treatment of Cancer (EORTC) Quality of Life Questionnaire-Core Questionnaire (QLQ-C30), EORTC Item Library 46 single item (IL46) and the EORTC Gastrointestinal Neuroendocrine Tumor module (QLQ-GI.NET21). Finally, 1:1 cognitive debriefing interviews with six patients with epNEC across the US, Germany and Italy assessed the content validity of PROs.

RESULTS

Clinicians reported several physical functioning (PF) impairments in patients with epNEC, driven by disease burden and treatment related toxicities (n=3/3) and confirmed the relevance of the selected PRO-CTCAE items (n=2/2). Focus groups expanded findings by highlighting major impacts on daily functioning and independence, and confirmed the relevance of all other PROs, except the QLQ-GI.NET21, which was then excluded due to

low relevance in epNEC (n=6/6). Patient interviews (n=5/6) confirmed the comprehension and relevance of the QLQ-C30 PF items, however, recall periods were interpreted inconsistently with typically around half of the participants (n=3/6) thinking of the current time. IL46 performed well for comprehension (n=4/5) and relevance (n=5/5) with a “past week” recall period (n=4/4). Other QLQ-C30 domains were considered relevant (n= 6/6 for most items). PRO-CTCAE items were easy to comprehend for the two participants who debriefed them.

CONCLUSIONS

Collectively, the clinician interviews, focus group and patient interviews confirm the high symptom and impact burden of epNEC and support the content validity (comprehension, relevance, and recall period) of the QLQ-C30 and IL46 and the comprehension of the PRO-CTCAE in epNEC.

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