Keywords: DeFi study data were used to assess the psychometric properties of the GODDESS tool. The GODDESS tool was developed by Memorial Sloan Kettering Cancer Center (MSKCC) and Desmoid Tumor Research Foundation (DTRF) using best practices and is the first disease-specific PRO instrument for desmoid tumors. Based on multiple rounds of DT patient interviews, the final version of the GODDESS tool was created, which is a 28-item questionnaire that was developed according to the US Food and Drug Administration guidance on developing PRO tools for a disease-specific PRO instrument (Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims, December 2009). BACKGROUND

GOunder/Desmoid Tumor Research Foundation DEsmoid Symptom/Impact Scale (GODDESS): Psychometric Properties and Clinically Meaningful Thresholds as assessed in the Phase 3 DeFi Randomized Controlled Clinical Trial

METHODS

- Analysis of patient responses to the GODDESS tool was conducted using blinded data from DeFi, a placebo-controlled, Phase 3 trial of nivolumab in adults with DT, aggressive fibromatosis (NCT03785964).
- The GODDESS tool was created, which is a 28-item questionnaire that was developed according to the US Food and Drug Administration guidance on developing PRO tools for a disease-specific PRO instrument (Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims, December 2009).

RESULTS

- The GODDESS tool was developed by Memorial Sloan Kettering Cancer Center (MSKCC) and Desmoid Tumor Research Foundation (DTRF) using best practices and is the first disease-specific PRO instrument for desmoid tumors.
- Based on multiple rounds of DT patient interviews, the final version of the GODDESS tool was created, which is a 28-item questionnaire that was developed according to the US Food and Drug Administration guidance on developing PRO tools for a disease-specific PRO instrument (Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims, December 2009).

OBJECTIVE

- DeFi study data were used to assess the psychometric measurement properties and clinically meaningful change thresholds (MCT) of GODDESS and to establish GODDESS as a fit-for-purpose PRO tool for the evaluation of symptoms and impact of patients with DT.

RESULTS

- GODDESS DESMOID TUMOR SYMPTOM SCALE (DTSS)
- GODDESS DTIS consists of 11 questions that assess the severity of key symptoms and include:
  - Pain, fatigue, swelling, muscle weakness, difficulty moving (items 1-7):
    - A question referring to tumor location (item 8); and
    - Intra-abdominal specific signs/symptoms (items 9-11) administered only to those reporting intra-abdominal tumor location in item 8.

- DTSS items 1-7 and 9-11 are evaluated on an 11-point numeric rating scale (NRS) from 0 to 10 to measure severity from “none” to “as bad as you can imagine”.

- Higher scores indicate more severe symptom burden.

GODDESS TOOL DEVELOPMENT

- Step 1: Identify DT-specific questions and develop draft PRO tool
- Step 2: Test measurement properties and confirm it is a fit-for-purpose tool.

CONCLUSIONS

- The GODDESS tool comprises two scales (DTSS and DTIS), with the following domains:
  - DTSS total symptom score, DTSS pain domain, DTSS intra-abdominal domain, and extra-abdominal domain;
  - DTIS physical functioning impact, DTIS sleep impact, and DTIS emotional impact.

- The GODDESS results were consistent with other well-known PRO tools (e.g., BPI-SF, PROMIS-PF, and EORTC QLQ-C30) where expected; this provides confidence that the proposed GODDESS domains measure the concepts they were hypothesized to measure.

- Moderate to strong correlations of similar concepts from other PRO tools:
  - For example:
    - DTSS Pain Domain and BPI-SF Worst Pain (strong);
    - DTSS Pain Domain and EORTC QLQ-C30 Pain subscale (strong);
    - DTSS Intra-abdominal Domain and EORTC QLQ-C30 Emotional Functioning (moderate).

- Other Relationship - Desmoid Tumor Research Foundation.

REFERENCES

3. SpringWorks Therapeutics, Stamford, CT, USA, provided funding to IQVIA conduct this study and was involved in reviewing this poster.
4. SpringWorks Therapeutics, Stamford, CT, United States; IQVIA, Patient-Centered Solutions, Athens, Greece; IQVIA, Patient-Centered Solutions, Paris, France.

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