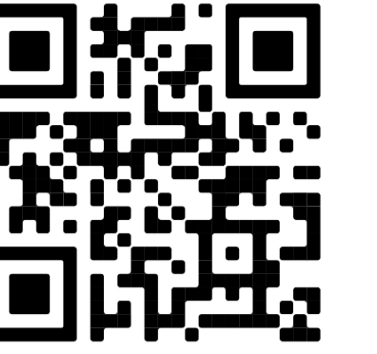


The Association of Pain With Function for Adult Patients With Desmoid Tumor: Findings From the Desmoid Tumor Research Foundation Natural History Study (2025)

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BACKGROUND

- Desmoid tumors (DT; aggressive fibromatosis) are rare, intermediate (locally aggressive), soft-tissue tumors that infiltrate surrounding structures, and can be life-threatening when vital organs are impacted¹⁻³
- Pain represents a key clinical symptom for adult patients with DT and has been reported to be a prognostic factor for poor outcomes, including potentially negative effects on a patient's function (eg, sleep, physical functioning, or emotional impact)^{4,5}
- A patient-reported outcome (PRO) tool (GUnder/DTRF DEsmoid Symptom/Impact Scale, GODDESS[®]) was developed and validated to evaluate DT-specific symptoms and impacts, including pain and function⁶
- The Desmoid Tumor Research Foundation (DTRF) Patient Registry and Natural History Study is part of the National Organization for Rare Disorders (NORD) IAMRARE program and has been collecting data since 2017⁷
 - The DTRF Natural History Study includes 15 surveys; 7 surveys were included to describe the symptoms through different clinical stages of rare disorders, and 6 were designed by the DTRF Natural History Study investigators for use in patients with DT⁸

OBJECTIVE

- To describe the real-world association of patient-reported, DT-specific pain with function using the DT-specific GODDESS[®] PRO tool

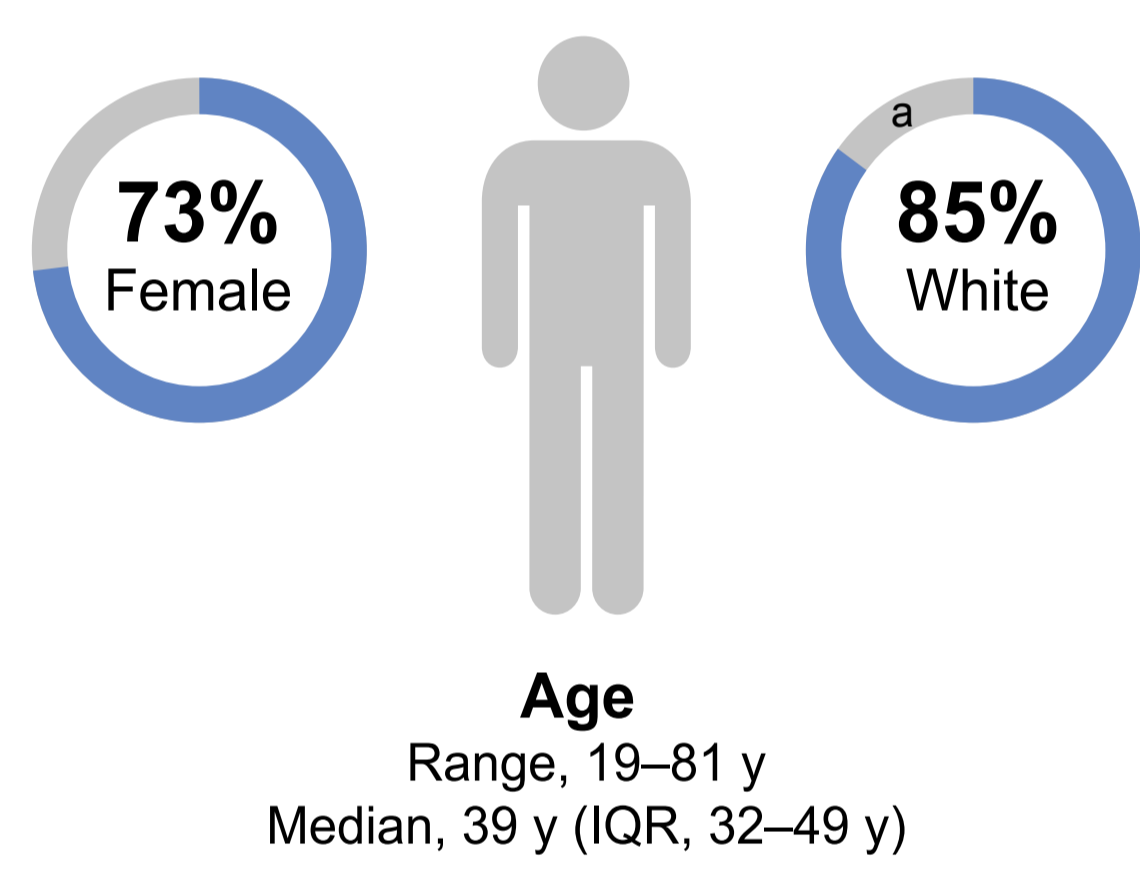
RESULTS

Patients and DT Characteristics

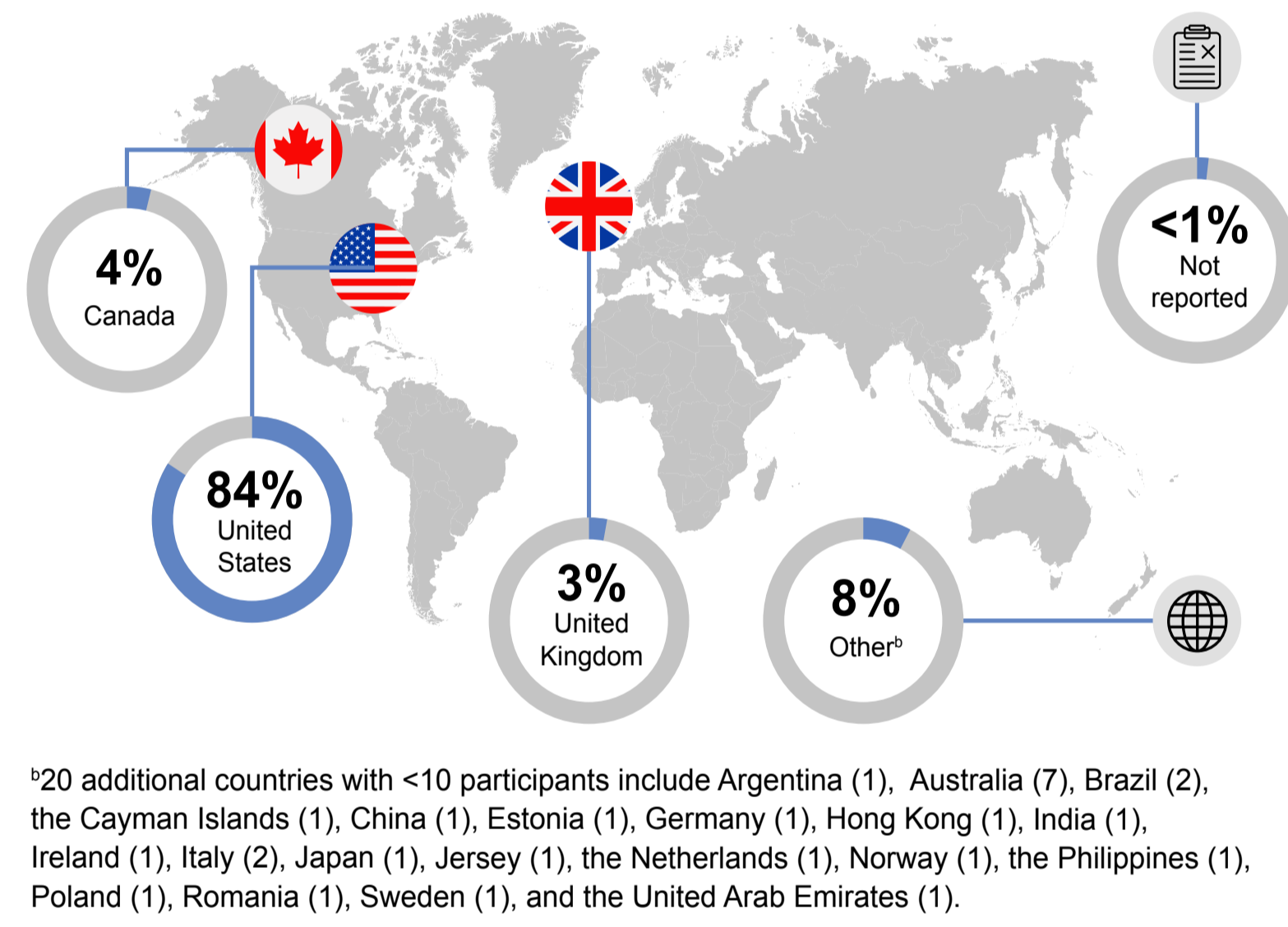
- In total, 335 patients completed the pain and function survey via the GODDESS[®] PRO tool (Figures 2 and 3)

Figure 2. Patient demographics (N=335)

A. Patient demographics



B. Patient country of residence

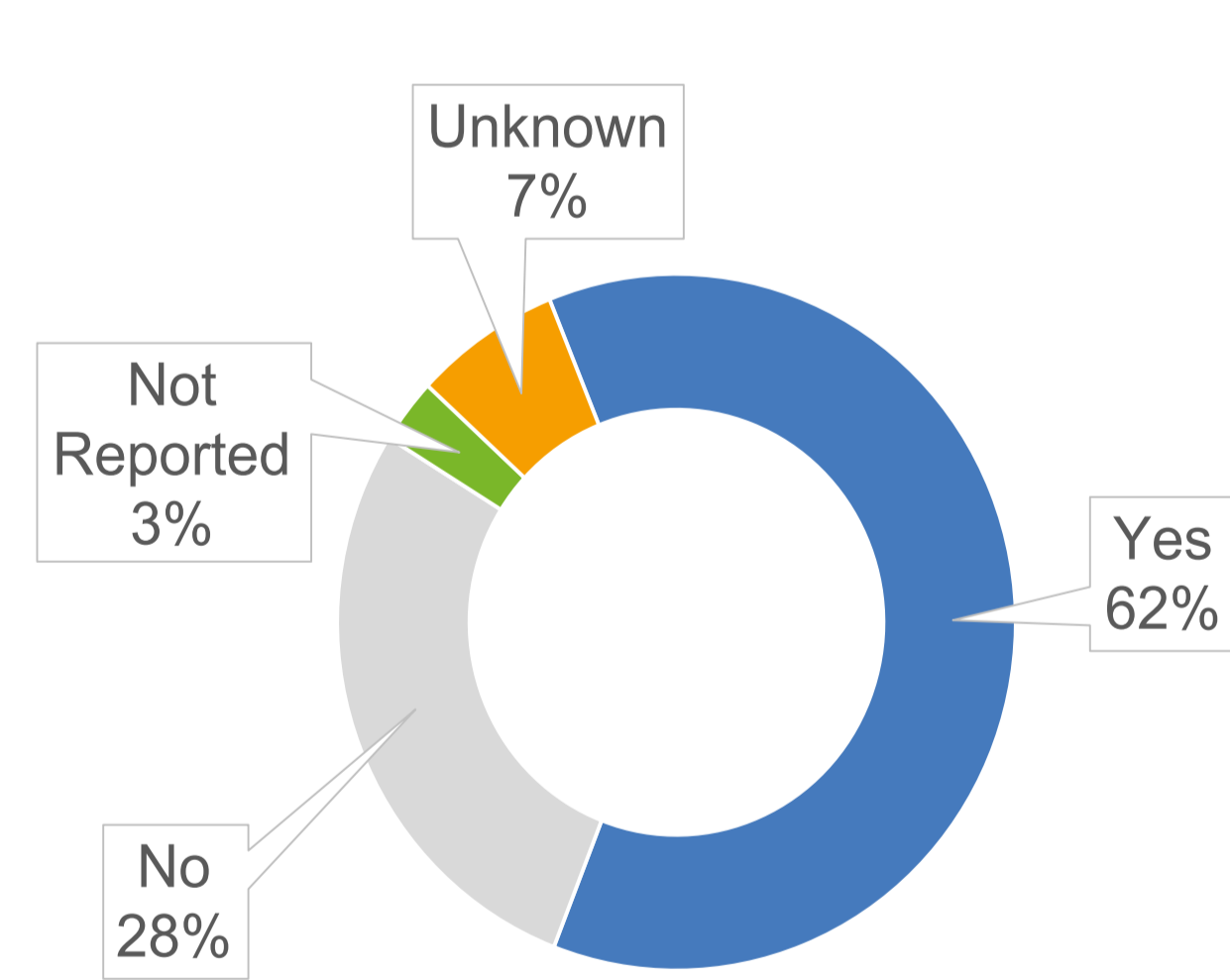


*Asian 2%; Black or African American 2%; American Indian or Alaska Native 1%; Native Hawaiian or Pacific Islander <1%; Other 3%; not reported 8%. IQR, interquartile range.

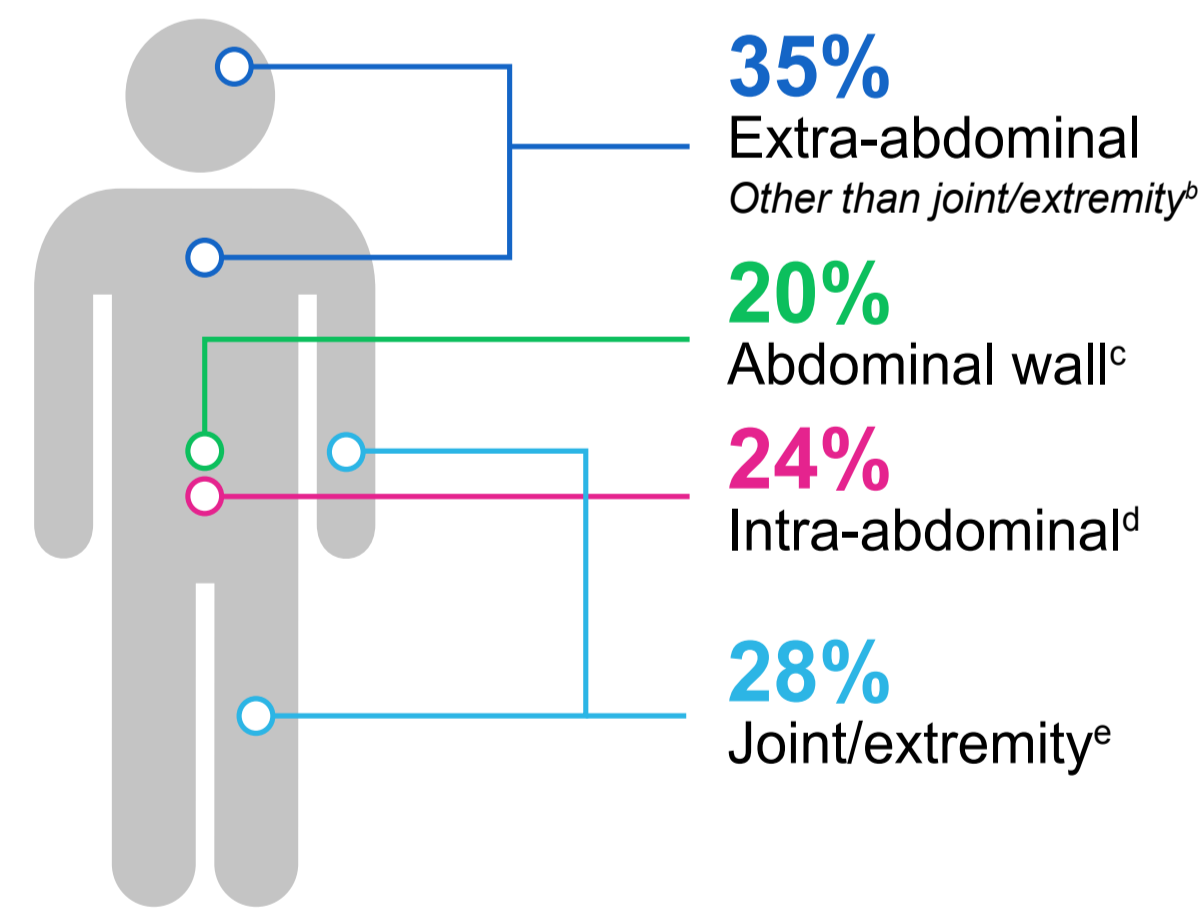
*20 additional countries with <10 participants include Argentina (1), Australia (7), Brazil (2), the Cayman Islands (1), China (1), Estonia (1), Germany (1), Hong Kong (1), India (1), Ireland (1), Italy (2), Japan (1), Jersey (1), the Netherlands (1), Norway (1), the Philippines (1), Poland (1), Romania (1), Sweden (1), and the United Arab Emirates (1).

Figure 3. Patient tumor characteristics (N=335)

A. Current presence of desmoid tumor



B. Desmoid tumor location^a

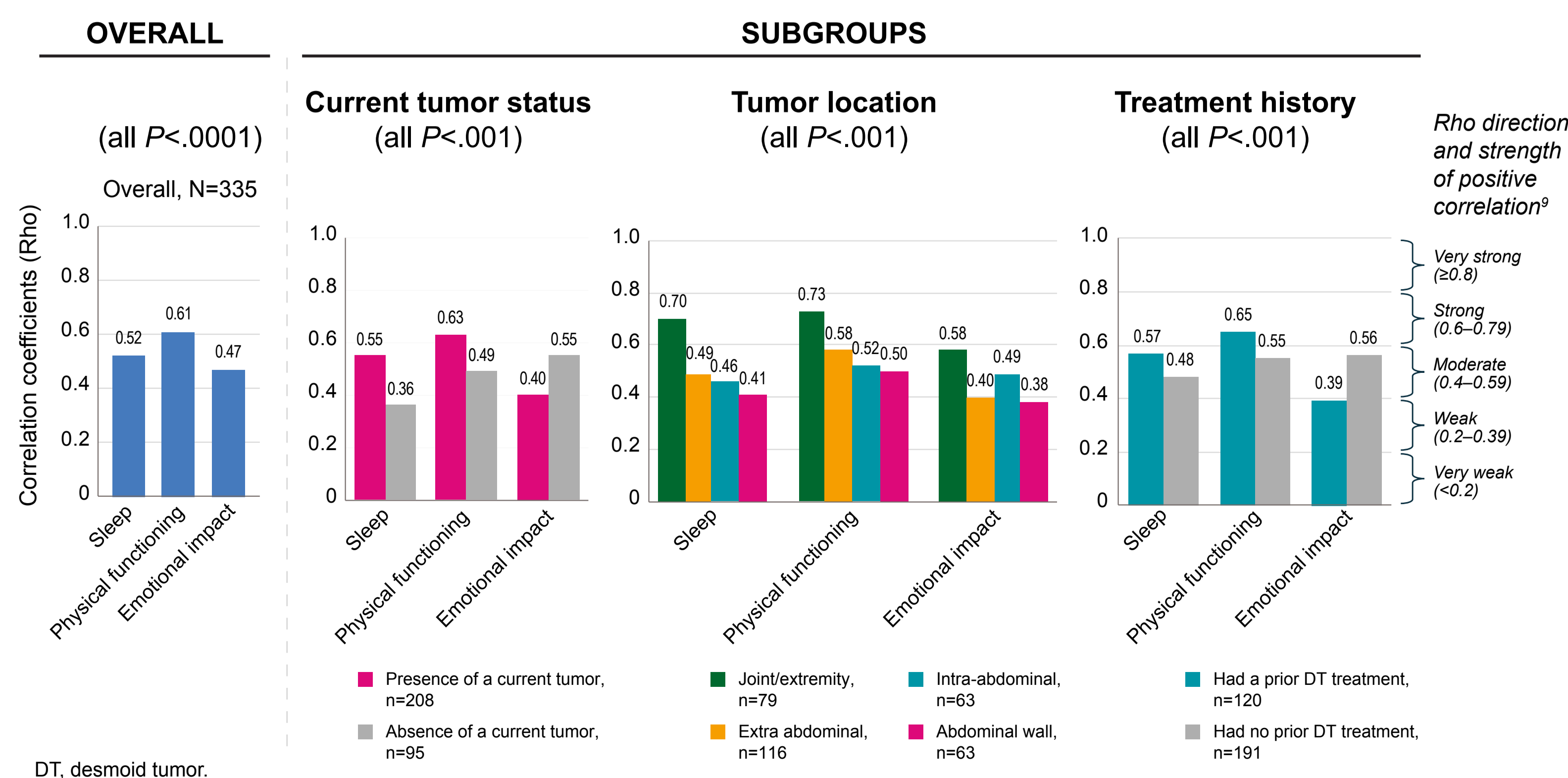


^aSome participants reported >1 tumor location; therefore, percentages do not total 100%. ^bRefers to the head/neck, chest wall, and other locations. ^cRefers to a superficial location on the stomach muscle. ^dRefers to locations deep in the abdomen and involving the bowels, kidney, and/or pelvis. ^eRefers to the hips, shoulders, arms, hands, feet, and legs.

Correlation of Pain With Function

- Overall, a significant positive correlation was identified between scores of the DTSS pain domain and all DTIS function domains ($P<.0001$) with coefficients (Rho) ranging from 0.47–0.61 (moderate to strong; Figure 4 overall)
- Moderate to strong positive correlations between DTSS (pain) scores and DTIS (impact) functional domain scores were also observed in all subgroups of patients (all $P<.001$; Figure 4 subgroups)

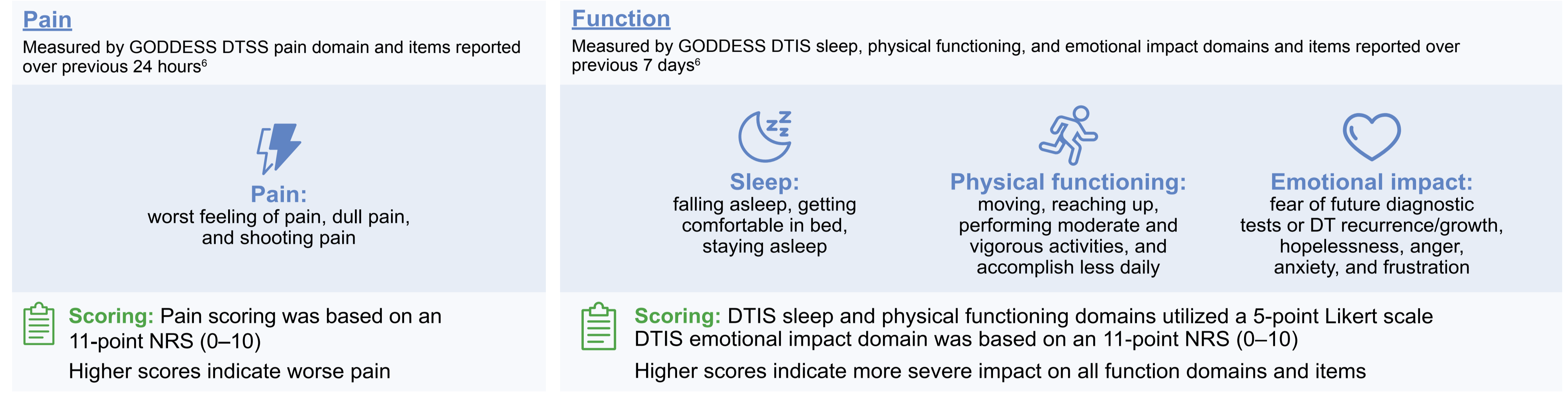
Figure 4. Correlation coefficients (Rho) between pain domain score and function domain scores



METHODS

- Data were obtained from the global, survey-based, DTRF Natural History Study during September 2017 to August 2023. This analysis captured the data from the most recently submitted survey for patients aged ≥ 18 years who spoke and read English
- The GODDESS[®] PRO tool includes the Desmoid Tumor Symptom Scale (DTSS) and Desmoid Tumor Impact Scale (DTIS; Figure 1)

Figure 1. Pain and Function Measurements

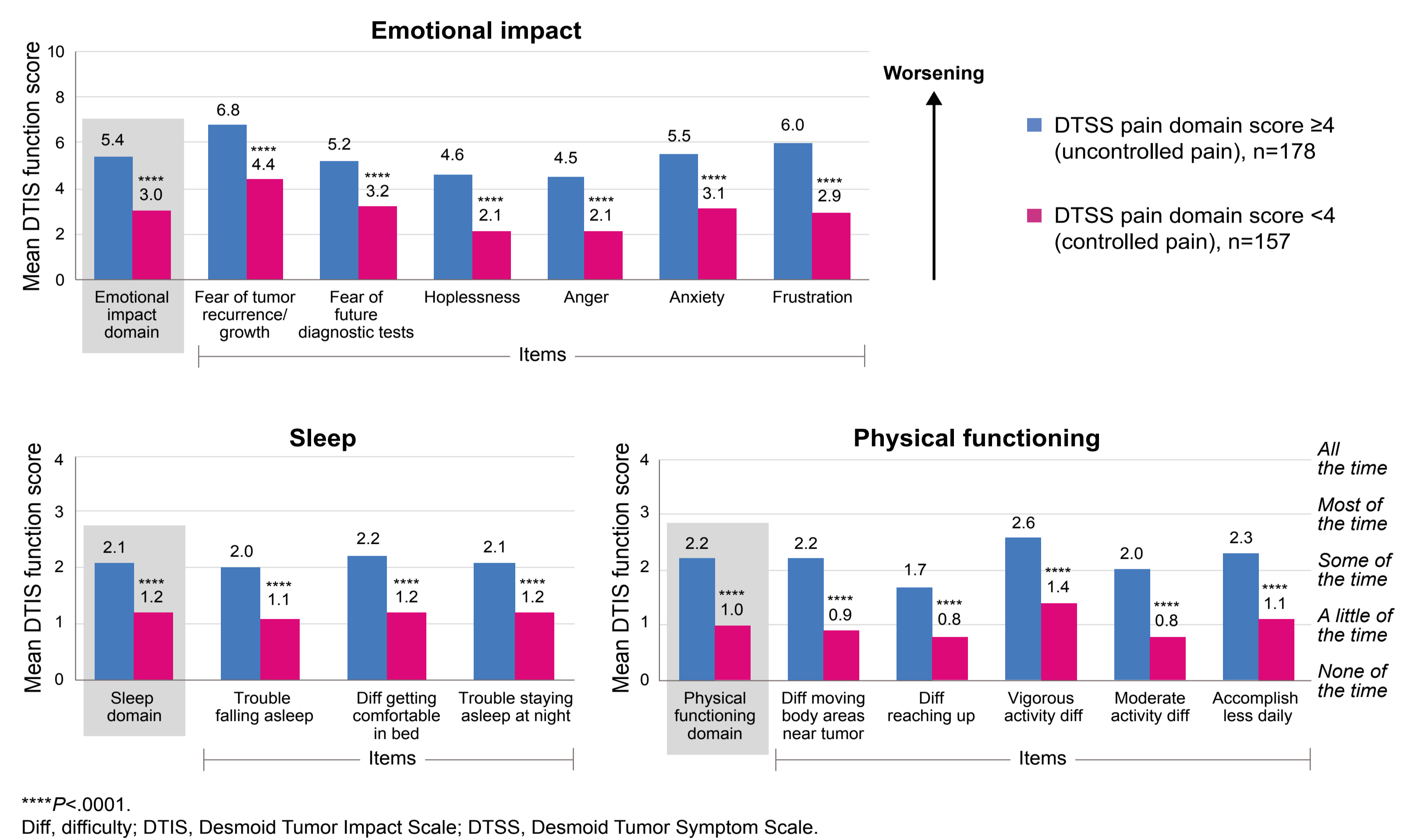


DTIS, Desmoid Tumor Impact Scale; DTSS, Desmoid Tumor Symptom Scale; NRS, numerical rating scale.

Impact of Pain on Function

- Overall, patients with uncontrolled pain had significantly higher (worse function) mean DTIS function domain scores vs patients with controlled pain ($P<.0001$) for sleep, physical functioning, and emotional impact (Figure 5)
 - Patients with uncontrolled pain (vs controlled pain) had an approximately 1-point higher mean score (clinically meaningful between-group difference, meaningful clinical threshold [MCT], 0.5 points for sleep and physical functioning⁶) on the 5-point Likert scale (0–4) across all DTIS sleep and physical functioning domains and items
 - Patients with an uncontrolled pain (vs controlled pain) had an approximately 2-point higher mean score (MCT, 2.0 points for emotional impact⁶) on the 0–10 scale across DTIS emotional impact domain and items

Figure 5. Impact of DTSS pain on DTIS function domain and item scores



**** $P<.0001$. Diff, difficulty; DTIS, Desmoid Tumor Impact Scale; DTSS, Desmoid Tumor Symptom Scale.

CONCLUSIONS

- The DTRF Natural History Study data show that DT-related pain correlates with the functional impact on patients' lives for DT-related sleep, physical functioning, and emotional impact
- Patients with uncontrolled pain (pain domain scores ≥ 4) had clinically meaningful worse scores on sleep, physical functioning, and emotional impact compared with patients with controlled pain (pain domain scores < 4)
- Patients with pain can experience negative effects across multiple dimensions of their daily life, including moving, reaching up, performing moderate and vigorous activities, accomplishing less daily, sleep, fear of future diagnostic tests or DT recurrence/growth, hopelessness, anger, anxiety, and frustration
- Because the GODDESS[®] DTSS and DTIS surveys solicit patients' responses based on the previous 24 hours and 7 days, respectively, the exact temporal coupling of pain with function cannot be fully characterized
- Successful care of patients with DT requires a multidisciplinary approach to effectively reduce pain. This key treatment goal should be prioritized in the clinical management of DT, which can support further functional improvements in sleep, physical functioning, and emotional impact

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DISCLOSURES

A. Lucas has no conflicts of interest to declare.

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