

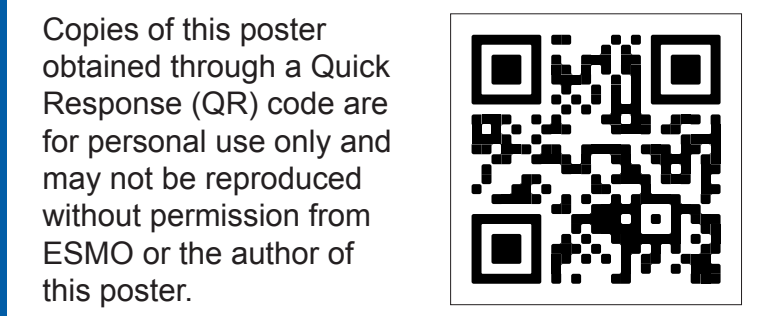
Impact of Desmoid Tumors on Patients' Quality of Life

Desmoid Tumor Research Foundation Natural History Study

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BACKGROUND

- Desmoid tumors (aggressive fibromatosis) are rare, locally aggressive, and potentially morbid soft-tissue tumors that infiltrate surrounding structures but rarely metastasize; in rare cases when vital organs are impacted, desmoid tumors can be life-threatening¹⁻³
- Patients may experience high symptom burden, including pain, impaired physical functioning, and fatigue, that can cause negative physical, emotional, and social impacts on daily activities, ultimately impacting their quality of life (QoL)¹⁻⁴
- Generic patient-reported outcome (PRO) tools have been used to evaluate QoL in patients with desmoid tumors but do not assess specific desmoid tumor symptoms and impact⁵

OBJECTIVE

- To describe real-world, patient-reported symptom severity and impact of desmoid tumors on QoL using a desmoid tumor-specific PRO tool

METHODS

- Data from the global, survey-based Desmoid Tumor Research Foundation (DTRF) Natural History Study were collected from Sept 2017 to Aug 2023
- The fifteen surveys included in the study were designed to describe the symptoms through different clinical stages of rare disorders (developed from templates produced by the National Organization for Rare Disorders [NORD]), and specifically desmoid tumors (designed by the DTRF Natural History Study investigators)^{6,7}
- Surveys were completed by adult participants with desmoid tumors who speak and read English
- Symptom severity (over the previous 24 hours) and impact on patients' lives (over the previous 7 days) were assessed using the GOUnder/DTRF Desmoid Symptom Scale (GODDESS[®] DTSS) and GODDESS Impact Scale (DTIS), respectively, together referred to as the GODDESS PRO tool⁸
- Domains assessed in GODDESS DTSS⁸ include pain (ie, pain, dull pain, shooting pain), extra-abdominal symptoms (ie, swelling, muscle weakness, difficulty moving body areas near the tumor), and intra-abdominal symptoms (ie, nausea, abdominal pain, and feeling of fullness after beginning to eat); fatigue was assessed as a single item

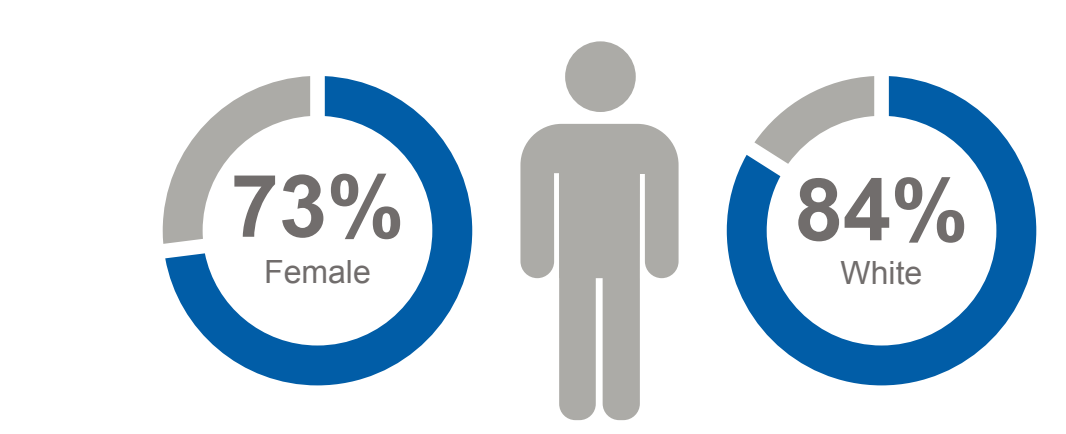
- A total symptom score was also calculated based on DTSS items using the following formula:
$$\frac{(\text{Pain} + \text{Dull Pain} + \text{Shooting Pain})}{3} + \frac{\text{Fatigue} + \text{Swelling} + \text{Muscle Weakness} + \text{Difficulty Moving}}{5}$$
- Domains measured in GODDESS DTIS⁸ include physical functioning (ie, moving body areas near the tumor, reaching up, performing moderate and vigorous activities, accomplishing less daily), sleep (ie, falling asleep, getting comfortable in bed, staying asleep), and emotional impact (ie, fear of future diagnostic tests or desmoid tumor recurrence/growth, hopelessness, anger, anxiety, frustration); avoidance because of appearance, difficulty reaching, and dissatisfaction with appearance were assessed as single items but are not reported in this analysis
- An 11-point scale was used to assess the DTSS domains and the DTIS emotional impact domain, where 0 represents no symptoms/impact and 10 represents the worst possible symptoms/impact; a 5-point Likert scale (0-4) was used to assess the DTIS physical functioning and sleep domains, where 0 means none of the time and 4 means all the time⁸
- Higher scores indicate worse symptoms or more severe impact⁸
- Analysis of variance (ANOVA) was used to assess for differences in mean scores between groups
- The N-values vary among analyses due to differing data completeness across survey questions

RESULTS

PARTICIPANT AND DESMOID TUMOR CHARACTERISTICS

- In total, 367 participants completed the GODDESS PRO tool

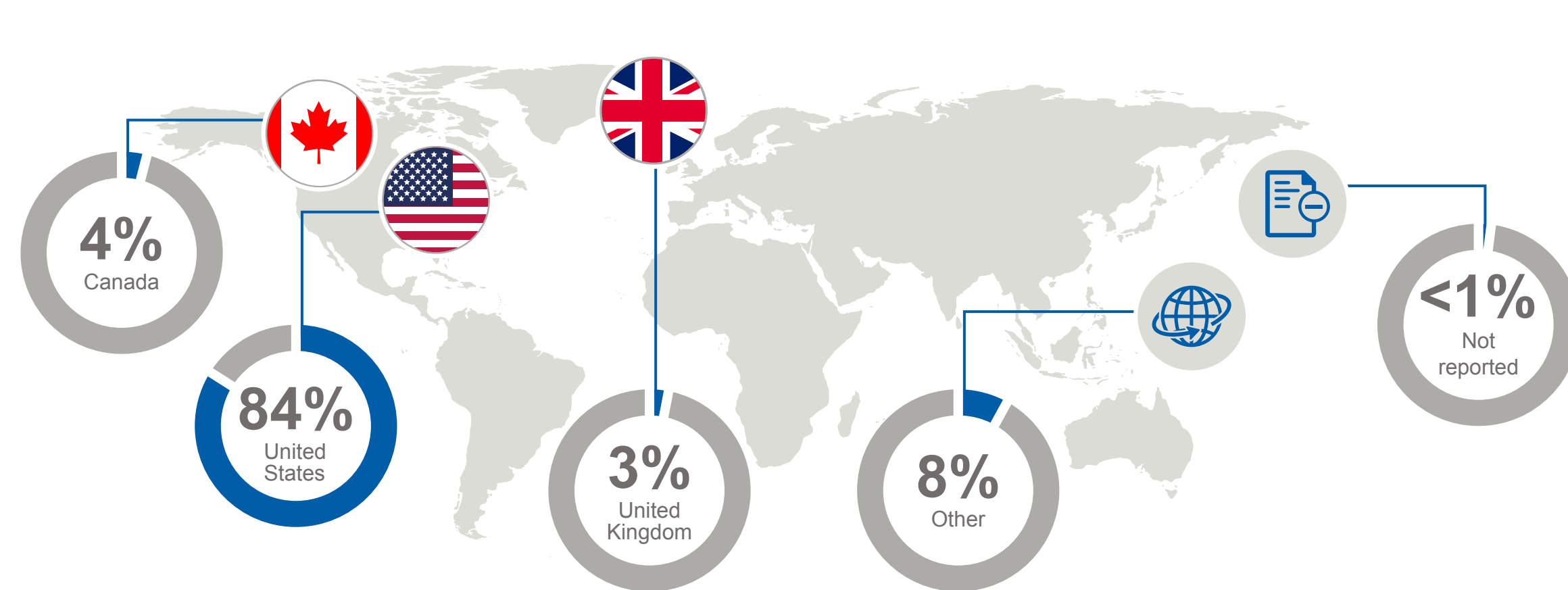
Participant Demographics (N = 367)



Age range 19-81 years Median 39 years (IQR, 32-48)

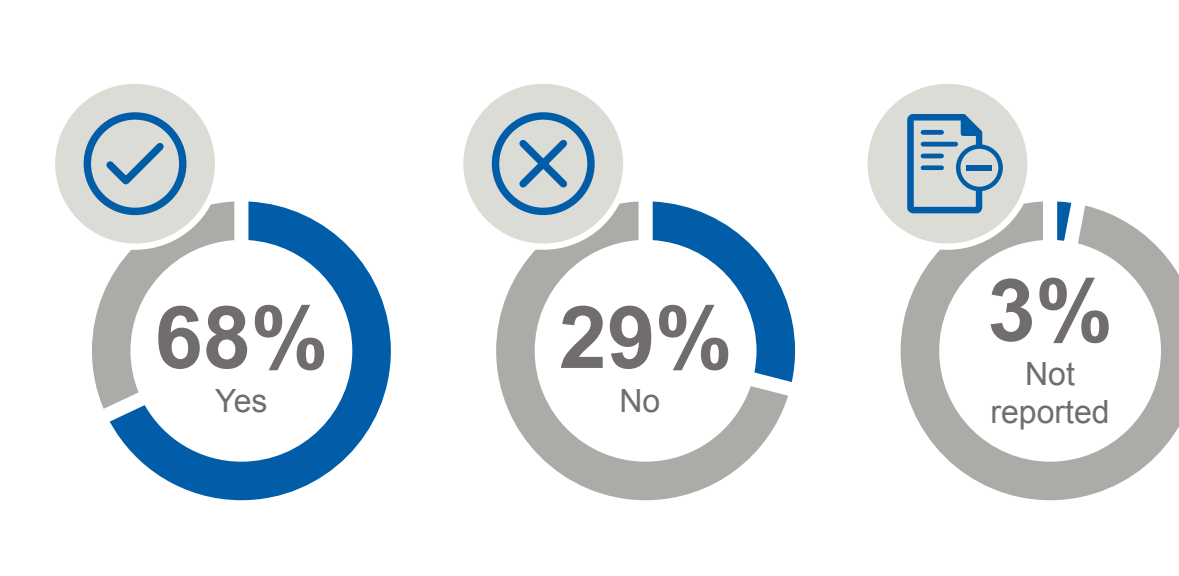
IQR, Interquartile range.

Participant Country of Residence^a (N = 367)

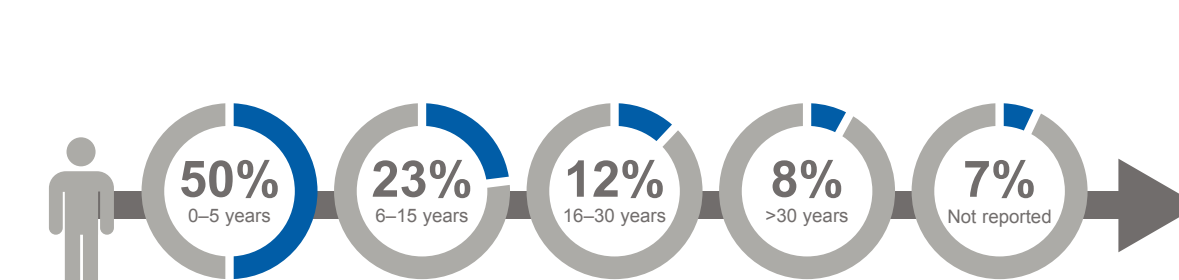


^a20 additional countries with <10 participants include Argentina (1), Australia (8), Brazil (2), the Cayman Islands (1), China (1), Estonia (1), Germany (1), Hong Kong (1), India (1), Ireland (1), Italy (2), Japan (1), Jersey (1), the Netherlands (1), Norway (1), the Philippines (1), Poland (1), Romania (1), Sweden (1), and the United Arab Emirates (1).

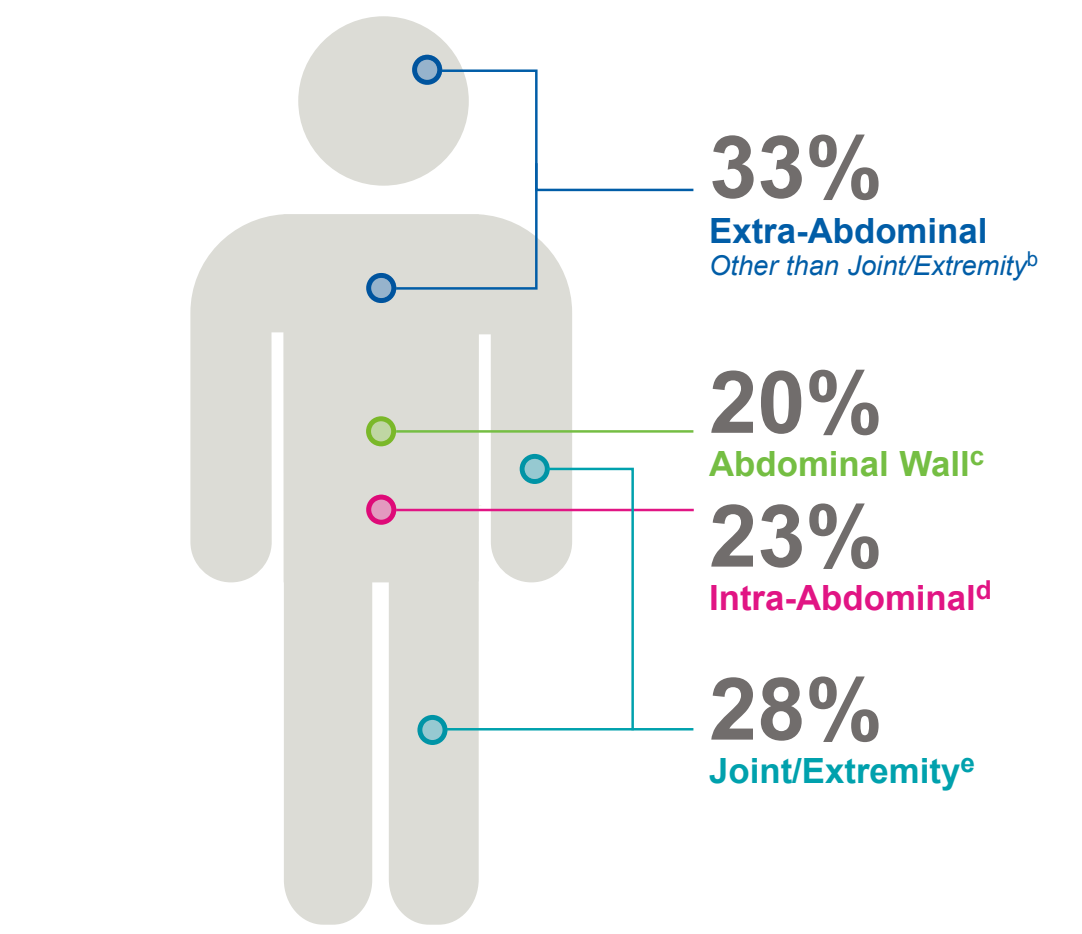
Presence of a Current Desmoid Tumor (N = 367)



Desmoid Tumor Duration (N = 367)



Desmoid Tumor Location^a (N = 367)



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

SYMPTOM BURDEN

- The DTSS mean total symptom score was significantly worse for participants with a current desmoid tumor versus those without (Figure 1a)
- Pain and extra-abdominal symptom domain scores, including item scores for pain, fatigue, swelling around tumor(s), and difficulty moving body areas near the tumor, were significantly worse for participants with a current desmoid tumor versus those without (Figure 1b and c)

IMPACT OF DESMOID TUMORS ON QOL

- Physical functioning and sleep domain scores, including item scores for difficulty doing moderate activities, difficulty moving body areas near the tumor, and getting comfortable in bed, were significantly worse for participants with a current desmoid tumor versus those without (Figure 2a and b)
- Participants with a current tumor (versus those without) experienced a significantly worse sense of hopelessness (Figure 2b)
- Fear of future diagnostic tests (overall mean, 4.3), fear of tumor recurrence/growth (overall mean, 5.6), and frustration (overall mean, 4.5) were commonly reported regardless of current tumor status

QOL OUTCOMES BY TUMOR LOCATION

- Participants with desmoid tumors in joint/extremity locations reported significantly worse scores for total symptoms, pain, and physical functioning (Figure 3) compared with other desmoid tumor locations

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EORTC QLQ-C30, European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire-Core Module; **GAD-7**, Generalized Anxiety Disorder Scale-7; **PHQ-9**, Patient Health Questionnaire-9.

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Figure 1. Symptom Scores (n = 353)

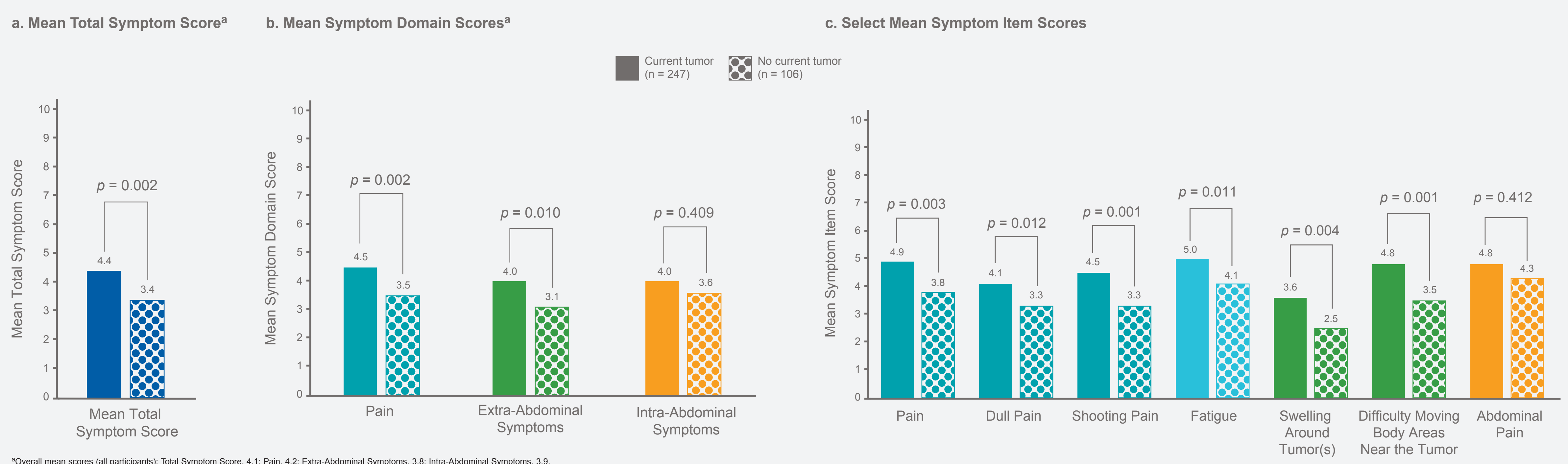


Figure 2. Impact Scores (n = 353)

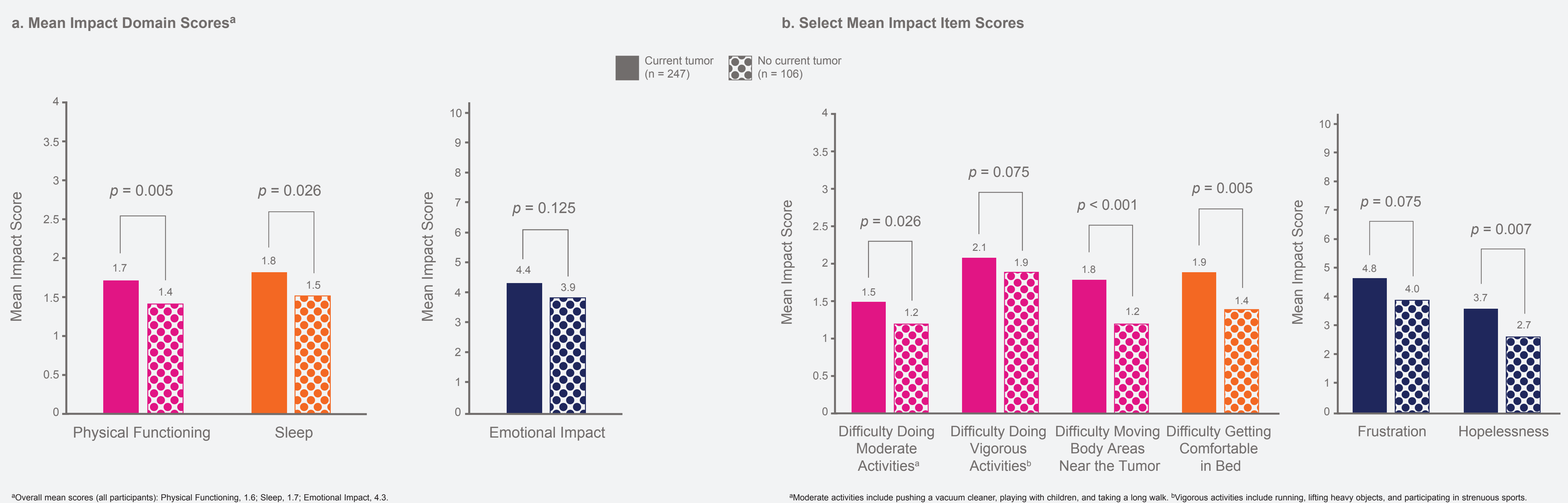
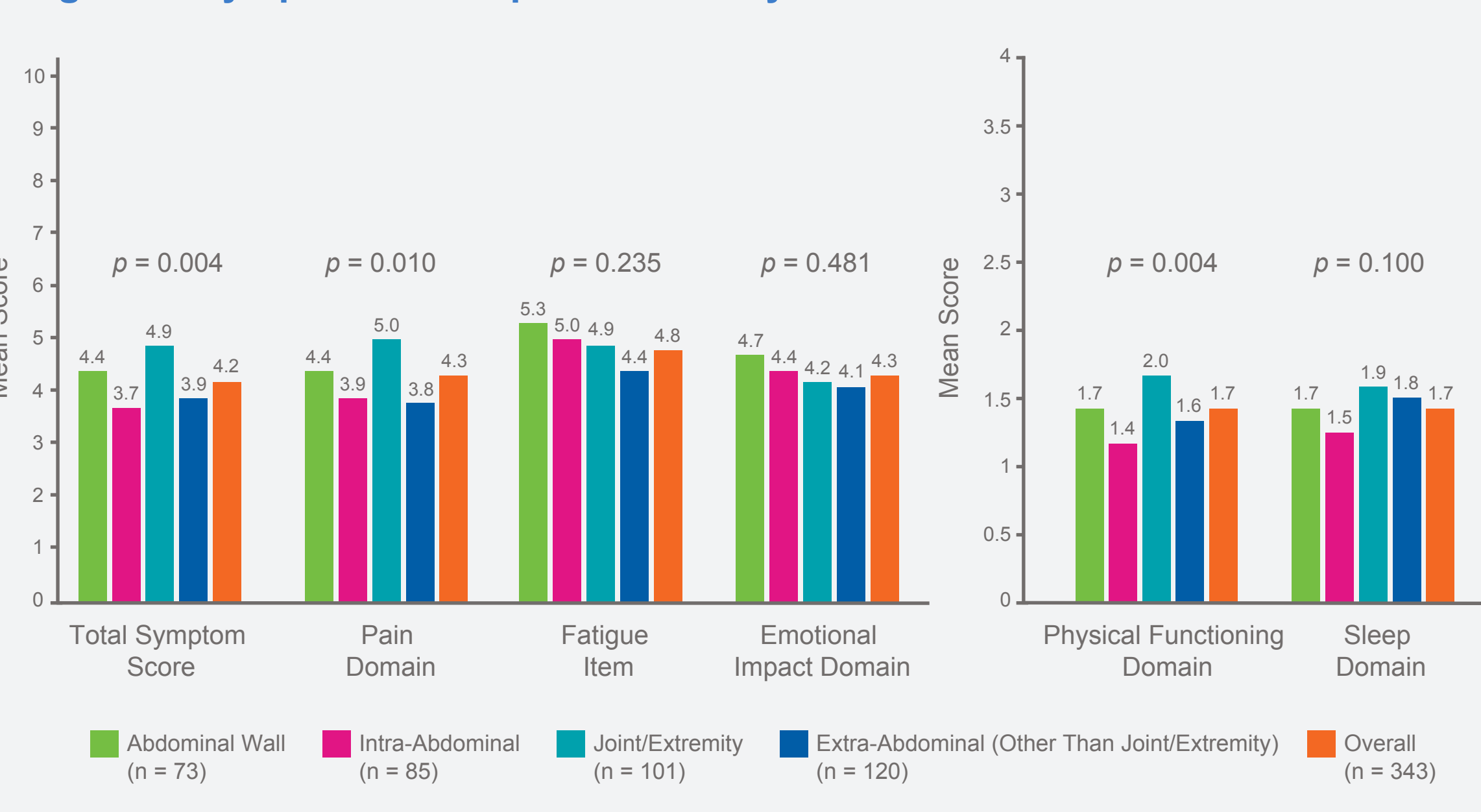


Figure 3. Symptom and Impact Scores by Tumor Location



CONCLUSIONS

- This analysis of DTRF registry data shows that desmoid tumors have a negative impact on patients' lives, where patients with a current tumor experienced significantly worse pain, fatigue, physical functioning, and sleep than those without a current tumor
- Participants reported fear of desmoid tumor recurrence or continued growth, as well as frustration related to their situation regardless of current tumor status; this suggests that, in addition to physical needs, care for patients with desmoid tumors should focus on emotional impact
- As the DTSS and DTIS surveys solicit participant responses based on the previous 24 hours and 7 days, respectively, symptom severity and impact on QoL may have an association with treatment and not be solely attributable to the desmoid tumor itself
- Data collected in this analysis using the GODDESS PRO tool are similar to other published data collected using EORTC QLQ-C30, GAD-7, and PHQ-9, which show high symptom burden due to pain, fatigue, insomnia, anxiety, and depression⁵
- Effective care of patients with desmoid tumors depends on understanding the burden and impact of these tumors