An Assessment of Financial Burden and Its Association With Desmoid Tumor Specific Symptoms and Impact in Adult Patients With Desmoid Tumors: Desmoid Tumor Research Foundation Natural History Study

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BACKGROUND AND OBJECTIVE

- Desmoid tumors (DT; aggressive fibromatosis) are rare, soft-tissue tumors that can be locally aggressive, can infiltrate surrounding structures, and may be life-threatening when vital organs are impacted[1–3]
- DT frequently can impose a considerable financial burden upon patients and their families due to the cost of diagnosis and treatment, potential loss of employment, and other indirect expenses, such as travel and childcare[4,5]
- A cancer-specific financial questionnaire of Patient-Reported Outcomes for Fighting Financial Toxicity (PROFFIT) was developed and validated to measure financial toxicity (FT) in patients with cancer[6,7]
- 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 2017[8]
  - The DTRF Natural History Study has a total of 15 surveys to collect and track participant responses over their course of the disease: 7 of the surveys report symptoms, and 6 of these 7 surveys for patient-reported outcomes were designed specifically for participants with DT[9]
- The objective of this analysis was to evaluate the patient-reported financial burden of DT and its association with DT-specific pain or function (measured by the GODDESS© PRO tool[10]) and FT score (using the PROFFIT tool)

METHODS

- Data were obtained from the global, survey-based DTRF Natural History Study from September 2017 to July 2024 using the most recently submitted survey completed by participants ≥18 years old who speak and read English
- Sixteen items were included in the PROFFIT survey. Seven outcome items were reported collectively as a composite FT score, and nine determinants were reported as single items (**Figure 1**) with higher scores indicating worse financial burden

Figure 1. PROFFIT tool: survey questionnaire

Outcome items (FT score)

1. Affords monthly expenses without difficulty
2. Illness reduced financial resources
3. Concern over future financial problems
4. Finances possibly affecting access to care
5. Less spending on leisure activities because of illness-related expenses
6. Less spending on essential goods because of illness-related expenses
7. Worry over ability to work because of illness

Determinant items (single items)

Medical expenses
8. Health costs coverage
9. Private medical exams pay
10. Additional medicines pay
11. Additional treatment pay

Transportation
12. Long travel for treatment
13. Expensive travel for treatment

Support from health system
14. Medical staff support
15. Hospital administration support
16. Staff communicate well

FT, financial toxicity; PROFFIT, Patient-Reported Outcomes for Fighting Financial Toxicity.

- All financial toxicity scores were normalized to a 0–100 scale, where a score of 100 indicates the highest toxicity
  - FT score (outcome items #1–7) was calculated by the following steps: 1) reverse the score for item #1 according to the formula $X_{1,reverse} = 5\text{-}X_1$, where X_1 is the response given to item #1; 2) calculate the FT score according to the formula $\frac{X_{1,reverse}+X_2+X_3+X_4+X_5+X_6+X_7-Y}{3\times Y}\times 100$, where X is the response given for each item, and Y is the number of items with a valid response. At least 4 valid responses are needed to calculate the FT score
  - FT determinant item scores (items #8–16) were calculated by the following steps: 1) the scores for items #8, #14, #15, and #16 were calculated using the formula $\frac{4-X_j}{3}\times 100$, where X is the response given, and j is the item (#8, #14, #15, or #16); 2) the scores for items #9, #10, #11, #12, and #13 were calculated using the formula $\frac{X_j-1}{3}\times 100$, where X is the response given, and j is the item (#9, #10, #11, #12, or #13)
- Median FT scores were compared using a Mann–Whitney test for the presence vs absence of a current DT or a prior history of DT treatment. A Kruskal–Wallis test was used for the analysis of DT location subgroups[7]
- The association of FT with DT-specific pain (measured by GODDESS© DTSS; **Figure 2**) or function (measured by GODDESS© DTIS; **Figure 2**)[10] was assessed by linear regression

Figure 2. Pain and function measurements

Pain

Measured by GODDESS DTSS pain domain and items reported over previous 24 hours[10]

Pain: worst feeling of pain, dull pain, and shooting pain

Function

Measured by GODDESS DTIS sleep, physical functioning, and emotional impact domains and items reported over previous 7 days[10]

Sleep: falling asleep, getting comfortable in bed, staying asleep

Physical functioning: moving, reaching up, performing moderate and vigorous activities, and accomplish less daily

Emotional impact: fear of future diagnostic tests or DT recurrence/growth, hopelessness, anger, anxiety, and frustration

Scoring: Pain scoring was based on an 11-point NRS (0–10)
Higher scores indicate worse pain

Scoring: DTIS sleep and physical functioning domains utilized a 5-point Likert scale
DTIS emotional impact domain was based on an 11-point NRS (0–10)
Higher scores indicate more severe impact on all function domains and items

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

RESULTS

Participants and DT characteristics

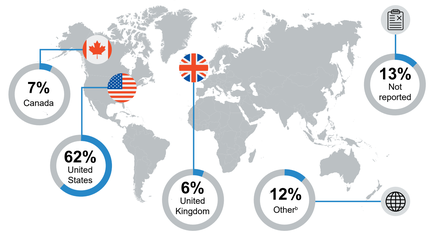
Figure 3. Participant demographics and country of residence (N=107)

A. Participant demographics

Age
Range, 20–81 y
Median, 44 y (IQR, 36–57 y)

[a]Asian 4%; Black or African American 1%; Native Hawaiian or Pacific Islander 1%; Other 3%; not reported 25%.
IQR, interquartile range.

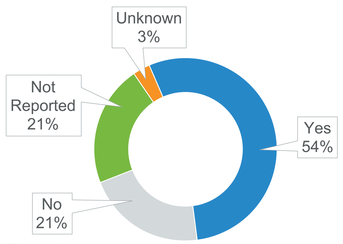
B. Participant country of residence



[b]11 additional countries with <5 participants include Australia (2), Botswana (1), Brazil (1), Germany (1), Hong Kong (1), India (1), Italy (2), the Netherlands (1), the Philippines (1), Sweden (1), and the United Arab Emirates (1).

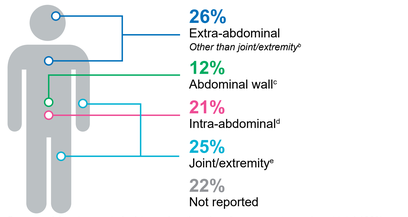
Figure 4. Participant tumor characteristics (N=107)

A. Current presence of DT



DT, desmoid tumor.

B. DT location[a]

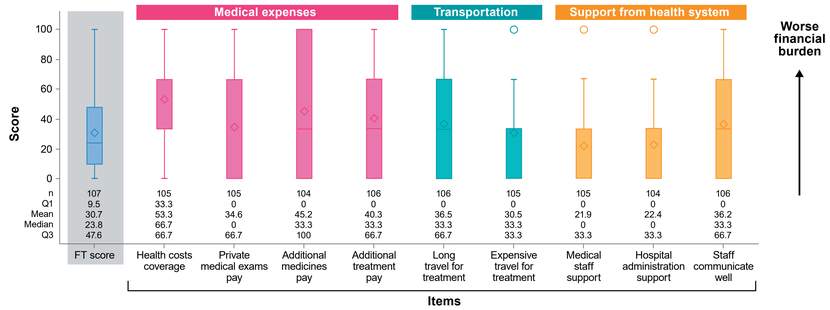


[a]Some participants reported >1 tumor location; therefore, percentages do not total 100%. [b]Refers to the head/neck, chest wall, and other locations. [c]Refers to a superficial location on the stomach muscle. [d]Refers to locations deep in the abdomen and involving the bowels, kidney, and/or pelvis. [e]Refers to the hips, knees, shoulders, arms, hands, feet, and legs.

Financial toxicity in participants with DT

- Overall, the median FT score was 23.8 (IQR, 9.5–47.6; scale, 0–100, **Figure 5 FT score**) for DT, which is similar to scores for malignancies[7] (**Table 1**). The median FT determinant scores were 33.3 for two transportation items, and ranged from 0 to 66.7 for medical expenses items, from 0 to 33.3 for support from the health system items (scale, 0–100; **Figure 5 items**)
- Participants with the presence of a current tumor (n=58) had a significantly higher (worse) FT score (median, 28.6 vs 14.3; *P*=.047) than those with the absence of a current tumor (n=23). No significant differences were observed in FT scores across tumor locations (median range, 19.0–33.3) or between participants with vs without prior treatment (19.0 vs 26.2; **Figure 6**)

Figure 5. Distribution of PROFFIT FT score (outcome items) and determinant items



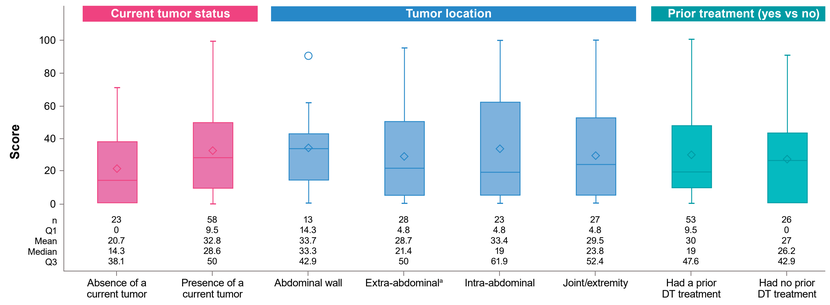
FT, financial toxicity; PROFFIT, Patient Patient-Reported Outcomes for Fighting Financial Toxicity; Q, quartile.

Table 1. FT score for DT and malignancies[a]

Type of tumor	n	FT score	
		Mean (SD)	Median (IQR)
DT	107	30.7 (27.3)	23.8 (9.5–47.6)
Thoracic	18	28.3 (28.9)	23.8 (4.8–48.8)
Breast	38	34.8 (23.5)	35.7 (14.3–52.4)
Gastrointestinal	71	29.2 (21.1)	28.6 (9.5–42.9)
Colorectal	25	22.3 (20.1)	14.3 (4.8–38.1)
Non-colorectal	46	32.9 (20.9)	33.3 (14.3–47.6)
Genitourinary	15	21.3 (16.6)	19.1 (4.8–33.3)
Gynecologic	20	25.2 (17.7)	23.8 (14.3–33.3)
Other	5	32.4 (13.6)	33.3 (19.1–45.2)

[a]DT values from Figure 5; malignancy values from De Vita et al.[7]
DT, desmoid tumor; FT, financial toxicity; IQR, interquartile range; SD, standard deviation.

Figure 6. Distribution of PROFFIT—FT scores (outcome items) by subgroups



[a]Extra-abdominal includes head/neck, chest wall, and other (which are not regrouped to the rest of the main categories). Each participant can report more than 1 tumor location.
DT, desmoid tumor; FT, financial toxicity; PROFFIT, Patient-Reported Outcomes for Fighting Financial Toxicity; Q, quartile.

Association of financial toxicity with DT-specific pain and function

- FT outcomes were significantly associated with the DTSS pain domain (*P*=.01) and the DTIS domains of sleep, physical functioning, and emotional impact (*P*<.0001)
- Some of the FT determinants were significantly associated with the DTIS domains of sleep, physical functioning, and emotional impact (*P*<.05; **Table 2**)

Table 2. Association of FT determinants with DTIS function domains

FT determinants (n=77)	Sleep (*p*-value)	Physical functioning (*p*-value)	Emotional impact (*p*-value)
Medical expenses			
Health costs coverage	NS	NS	NS
Private medical exams pay	<.05	<.05	<.05
Additional medicines pay	NS	<.05	<.05
Additional treatment pay	<.05	<.05	NS
Transportation			
Long travel for treatment	NS	NS	NS
Expensive travel for treatment	<.05	NS	<.05
Support from health system			
Medical staff support	NS	NS	NS
Hospital administration support[a]	NS	<.05	NS
Staff communicate well	NS	<.05	NS

[a]Hospital administration support, n=76.
FT, financial toxicity; NS, not statistically significant.

CONCLUSIONS

- These DTRF Natural History Study data demonstrate that DT can impose an FT comparable to, if not worse than, that experienced by patients with certain malignancies (**Table 1**), such as thoracic tumors, gastrointestinal colorectal cancer, genitourinary tumors, or gynecologic tumors[7]
  - Participants with the presence of a current tumor reported significantly higher FT scores than those with the absence of a current tumor, while no differences in FT scores were noted across tumor locations or between participants with a prior history of DT treatment vs without
- Financial burden for participants with DT can be driven by direct costs related to disease management and indirect costs, such as travel for care or support from the healthcare system; this burden can also be associated with DT-specific pain and functional limitations
- Understanding the impact of patient-reported financial burden in DT management and health outcomes is meaningful and can potentially help facilitate appropriate counseling and resource case management as well as improve treatment adherence and coordinate other psychosocial aspects of care

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DISCLOSURES

SZ, TB, and AO are employees of SpringWorks Therapeutics, Inc. and may have an equity or other financial interest in SpringWorks; AL, LH, and KM have no conflicts of interest to declare; YC is a consultant for SpringWorks Therapeutics.

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