Patient Burden of Illness Associated With Desmoid Tumors

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BACKGROUND

Desmoid tumors (DTs) are known as desmoid-type fibromatoses or aggressive fibromatoses—tumors that are locally aggressive, fibroblastic soft-tissue tumors that are characterized by infiltrative growth and a tendency to recur.1,2

DTs are associated with significant symptom and physical function burden that can impact patient overall health-related quality of life (HRQoL).3

The course of DT is unpredictable, as spontaneous regression, long-lasting stable disease, and disease progression can occur.2

OBJECTIVE

To review the epidemiology, clinical, hematologic, and economic burden, and available treatments for DT.

METHODS

• Literature database searches for English-language articles from November 2011 to November 2021 were conducted in PubMed, DIALOGUE, and the Cochrane Library.

• Articles on disease description and its presentation; epidemiology; clinical, hematologic, and economic burden; and treatment guidelines were identified.

• Conference proceedings published from 2015 to 2021 were also reviewed. In addition, the bibliographies of identified literature were scanned, and key studies were reviewed to identify additional seminal studies published before 2011.

RESULTS

EPILOGUE RISK FACTORS

• Family history of colorectal cancer (CRC) (patients with CRC who have APC mutations may have up to a 30% risk of developing DT),

• Hormonal contraceptives,

• Smoking,

• Trauma (DT develops following surgical trauma or injury),

• Use of oral contraceptives.

ECONOMIC BURDEN

No studies evaluating direct or indirect costs of patients with DTs were identified in the literature. Indirect resource utilization of patients with DTs is crucial for estimating the economic burden of the disease. The lack of studies investigating resource utilization and costs means that no study was identified, and no study evaluating the direct or indirect costs of patients with DT was identified.

Within the last 3 years before the index date, patients with DT had, on average, 1.1 average and 7.1 annual visits and 7.6 days in the hospital compared with an average of 0.2 average and 0.9 visits and 8.4 days in the hospital in the comparison cohort as a whole.

DT impacts employment and job productivity. In a survey conducted among French patients with DT, 36% stopped working, and 10% worked part time (Table 1).

GUIDELINES AND CURRENT TREATMENTS

There are currently no Food and Drug Administration–approved treatment options specifically indicated for patients with DT.

Because of the highly variable presentation and symptoms of DT, treatment requires a高低 individualized approach.

Two treatment guidelines provide general guidance on the management of DT: the National Comprehensive Cancer Network (NCCN)’s Guideline and the Desmoid Tumor Working Group (DTWG)’s Guideline.25 Figure 2 presents key recommendations for the management of DT.

LIMITATIONS OF THE LITERATURE

• The number of publications on epidemiology, hematologic burden, and economic burden was limited, which considerably impaired the assessment for burden of the disease. Specifically, no epidemiological studies in the US were identified, and no study evaluating the direct or indirect costs of patients with DT was identified.

CONCLUSIONS

• The substantial burden of illness of DT is related to mood, physical burden, and physical and functional limitations, and decreased quality of life (due to negative impacts on physical, emotional, and social domains). There is a high unmet need for treatments that target DT and are associated with improved quality of life for patients.

Table 1. Summary of Health-Related Quality of Life in Desmoid Tumors

<table>
<thead>
<tr>
<th>Study Design</th>
<th>Population</th>
<th>Key Findings</th>
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</table>
| Cross-sectional study | DT (n = 152) and healthy controls (n = 192) | Most frequent reported symptoms across tumor locations: 
- Distress (18%) 
- Pain (3%) 
- Depression (6%) 
- Anxiety (5%) 
- Fatigue (36%) 
Most frequent reported impacts on patients’ lives: 
- Fear (36%) 
- Sleep disturbance (27%) 
- Concern about lack of knowledge among healthcare providers (75%) |

Table 2. Management of Desmoid Tumors

<table>
<thead>
<tr>
<th>Treatment</th>
<th>Aim</th>
<th>Managing the treatment burden</th>
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<tbody>
<tr>
<td>Local resection</td>
<td>Resecting the tumor while preserving tissue and function</td>
<td>Requires multidisciplinary care and long-term follow-up</td>
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<tr>
<td>Radiotherapy</td>
<td>Reducing tumor size and improving symptoms</td>
<td>Associated with potential side effects</td>
</tr>
<tr>
<td>Chemotherapy</td>
<td>Managing aggressive tumors</td>
<td>Requires careful monitoring for toxicity</td>
</tr>
<tr>
<td>Proton beam therapy</td>
<td>Providing precise radiation dose</td>
<td>Available at specialized centers</td>
</tr>
<tr>
<td>Surgery</td>
<td>Providing definitive treatment for selected cases</td>
<td>Associated with surgical risks</td>
</tr>
</tbody>
</table>

REFERENCES


6. Articles on disease description and its presentation; epidemiology; clinical, hematologic, and economic burden; and treatment guidelines were identified.

7. Several risk factors for the development of DT are cited in the literature: family history of colorectal cancer (CRC) (patients with CRC who have APC mutations may have up to a 30% risk of developing DT), hormonal contraceptives, smoking, trauma (DT develops following surgical trauma or injury), and use of oral contraceptives.

8. Within the last 3 years before the index date, patients with DT had, on average, 1.1 average and 7.1 annual visits and 7.6 days in the hospital compared with an average of 0.2 average and 0.9 visits and 8.4 days in the hospital in the comparison cohort as a whole.

9. DT impacts employment and job productivity. In a survey conducted among French patients with DT, 36% stopped working, and 10% worked part time (Table 1).

10. The current DT guidelines provide general guidance on the management of DT: the NCCN’s Guideline and the DTWG’s Guideline. Figure 2 presents key recommendations for the management of DT.

11. The substantial burden of illness of DT is related to mood, physical burden, and physical and functional limitations, and decreased quality of life (due to negative impacts on physical, emotional, and social domains). There is a high unmet need for treatments that target DT and are associated with improved quality of life for patients.