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Desmoid fibromatosis in children and adolescents: A conservative approach to management

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Abstract

Purpose: Desmoid fibromatosis is associated with frequent recurrence and significant morbidity, but no metastases. To examine the impact of initial non-operative management on event-free survival (EFS) in children, we reviewed our institutional experience with this tumor.

Methods: We retrospectively reviewed our institutional database for pediatric cases of desmoid fibromatosis treated between 1970 and 2010. Survival was analyzed using the Kaplan–Meier method and log-rank test.

Results: Ninety-three patients were identified, with a median follow-up of 6 years. Median age at diagnosis was 16 years. Forty-seven patients presented with primary tumors, and forty-six had recurrent or progressing disease. Five-year OS was 100%, and 5-year EFS was 31.8%, with a median time to event of 1.48 years. There was no significant difference in 5-year EFS between patients who were managed expectantly and those who initially received treatment (21% versus 34%, $P=.09$). Sex, race, history of trauma, or familial adenomatous polyposis, multifocality, tumor size, tumor location, and resection status did not correlate with EFS.

Conclusion: Our findings support a conservative initial approach in the management of desmoid fibromatosis. In patients at risk for morbid procedures, upfront resection should be reserved for select tumors that demonstrate aggressive growth or cause serious symptoms.

Although desmoid fibromatosis does not metastasize, its natural history is characterized by aggressive growth and a tendency towards local invasion [1]. While surgical resection remains the primary treatment for these tumors, published recurrence rates in the pediatric population range from 23% to 83% [2–7], requiring patients to undergo multiple resections in pursuit of a cure. Some series have found that negative surgical margins are associated with lower rates of recurrence [2,3], but operative sequelae contribute to the overall morbidity of the disease [8]. Although chemotherapy and radiation

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have demonstrated treatment efficacy [6,9], each has its own risk profile.

Data from the adult literature suggest that some desmoid tumors can be managed non-operatively, with observation and close follow-up [10]. However, there are scant data in the literature on the impact of expectant management of desmoid tumors in the pediatric population. In this study, we retrospectively reviewed the records of pediatric patients with desmoid fibromatosis who were treated at our institution, an urban cancer center, over the past 40 years to determine the relative efficacy of a wait-and-see approach in the management of this complex disease.

1. Methods

With Institutional Review Board approval, we performed a retrospective review of our institutional pathology database to identify all patients under 21 years of age with desmoid fibromatosis treated between 1970 and 2010. A total of 96 patients with desmoid tumors were identified. Three patients were excluded from the study for incomplete follow-up; the remaining 93 patients were included in the final analysis. The electronic medical records of all patients were reviewed for data on demographics, past medical history, diagnosis, pathology, treatment, complications, and outcomes. A patient was defined as having undergone observation if there was a tissue diagnosis of desmoid tumor but the patient had no surgery or systemic therapy other than an NSAID.

Pathology accession numbers were obtained for all patients, and these were submitted for histopathologic review. Of the 93 patients included in the final analysis, 50 had samples available for review in the department of pathology. All reviewed pathology confirmed the existing chart diagnosis of desmoid tumor.

Overall survival (OS) and event-free survival (EFS) were calculated using the Kaplan–Meier method. The EFS analysis included calculations for the entire study population, as well as for patients aged 15 years and younger. An event was defined as radiographic or clinical tumor progression, tumor recurrence, or operative intervention for the management of existing disease. Factors included in the univariate analysis included sex, history of familial adenomatous polyposis (FAP) or trauma, pain on diagnosis, anatomic location of disease, intra-abdominal tumor, tumor size, observation, surgery, chemotherapy, radiation therapy, and resection status. The log-rank test was used to determine significance. A P value less than .05 was considered statistically significant.

2. Results

2.1. Patient demographics, tumor characteristics, and treatment

A total of 93 patients with desmoid fibromatosis were treated during the study period, with an age at diagnosis ranging from 2 months to 21 years (median, 16.1 years). Median follow-up for the group was 6.4 years (range, 11 months–25 years). The male-to-female ratio was 1.1:1. Tumor site distribution was as follows: 60 (65%) in the extremities, 15 (16%) intra-abdominal, 13 (14%) in the thoracic or abdominal wall, and 5 (5%) in the head and neck. Ten patients (11%) had a past medical history of FAP, and 17 (18%) had history of antecedent trauma (Table 1). Eighty-three patients (89%) underwent tumor resection at some point during treatment. Fifty-four patients (58%) required more than one operation during the course of their entire treatment; 10 patients (11%) had 4 or more operations.

Forty-seven patients presented to our institution with primary tumors, and 46 presented with recurrent or progressing tumors. For the entire study population, 15 patients were observed and 78 underwent a therapeutic intervention. Sixty-two patients had surgery as part of their management; adjuvant treatments included radiotherapy (n=11), NSAID treatment (n=3), systemic chemotherapy (n=4), and a combination of systemic therapy and radiotherapy (n=2). Of the 16 patients who received non-operative, therapeutic treatment, 14 had systemic therapy and 2 underwent cryoablation. A summary of treatments can be found in Table 2.

2.2. Survival analysis

Overall survival for the study population was 100% at 5 years. There were 2 disease-related deaths in the study group, both in patients with FAP, at 5 years and 10 years post-

Table 1 Patient demographic information and tumor characteristics (N=93).

Patient/disease parameter	Median value (range) or number of patients (%)
Age at diagnosis	16 y (range: 2 mo–21 y)
Sex	
Male	49 (52.6%)
Female	44 (47.3%)
Presenting symptom	
Mass	75 (81.6%)
Pain	22 (23.7%)
Limited ROM	4 (4.3%)
Limb length disparity	2 (2.2%)
GI symptoms	3 (3.2%)
History of FAP	10 (10.8%)
Tumor location	
Extremity	60 (65%)
Intra-abdominal	15 (16%)
Trunk	13 (14%)
Head and neck	5 (5%)
Tumor size	
<5 cm	25 (26.8%)
5 to 10 cm	28 (30.1%)
>10 cm	32 (34.4%)
Unavailable	8 (8.6%)

ROM, range of motion; FAP, familial adenomatous polyposis.