TITLE: A Patient Reported Outcomes of Treatments for Desmoid Tumors an International Natural History Study

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BACKGROUND AND OBJECTIVES: The Desmoid Tumor Research Foundation (DTRF) launched the natural history study (NHS) in 2017. At this time, there are no standard-of-care options for this rare sarcoma. The treatments, clinical descriptors, and the patient reported outcomes to pharmacologic agents are described here within.

METHODS: The web-based natural history study launched September 2017 in collaboration with the National Organization of Rare Disorders. It contains 15 surveys covering diagnostics, disease, treatment, care management, and quality of life. Treatment types included in the DTRF NHS were pharmacology, surgery, radiation, high-intensity focused ultrasound (HiFU), and active surveillance (watch and wait).

RESULTS: While surgery was once the primary intervention for desmoid tumor patients, the NHS participants reported that 47.6% had received active surveillance or no systemic treatment at diagnosis. This is most common for desmoid tumors located in abdominal wall (54/103; 52.4%). There were 87 reported cases of complete surgical resection, 38 incomplete resections, and 23 bowel resections. 9 amputations were reported; 8 participants reported recurrent disease following the removal of the limb. The non-surgical interventions, such as radiation and HiFU, were mostly described for participants with chest wall tumors (15 pts) and joints/extremities (10 pts). Many options for systemic therapies were described including sorafenib (44/284; 15.5%), sulindac (36/284; 12.7%), and anti-hormonal agents tamoxifen and toremifene (34/285; 10.9%) were described. Targeted agents, such as gamma secretase inhibitor, pazopanib, and sorafenib, were greater in the United States than the non-US country participants (21% vs 9%). Multiple lines of treatments were reported by 81 participants, surgery is greatest as the first intervention for all tumor locations (49/81, 60%), with the exception of those with head/neck tumors who received chemotherapy (6/11, 55%). Analysis has started to evaluate the efficacy of systemic treatments from these NHS data. The table describes the participant reported outcomes of anti-hormonal agents, chemotherapeutics, non-steroidal anti-inflammatories, and targeted agents. Both chemotherapies and targeted agents were reported to have 38.1% response rates from the participants with 34.3% and 23.8% of participants reported progressive disease on therapy, respectively.

CONCLUSION: Desmoid tumor NHS study participants reported the use of many treatment modalities demonstrating a range of frequency of use by tumor location and efficacy. Data collection through the DTRF NHS is ongoing.

	Tumor Shrinking/ Changing structure	Continued growth	Unknown Response
Anti-hormonal agents	8 (8.2%)	19 (18%)	26 (19.3%)
Chemotherapies	37 (38.1%)	36 (34.3%)	48 (35.6%)
Non-steroidal anti-inflammatories	15 (14.5%)	25 (23.8%)	49 (36.3%)
Targeted agents	37 (38.1%)	25 (23.8%)	12 (8.9%)