

#11560: Patient Reported Outcomes of Treatments for Desmoid Tumors: An International Natural History Study

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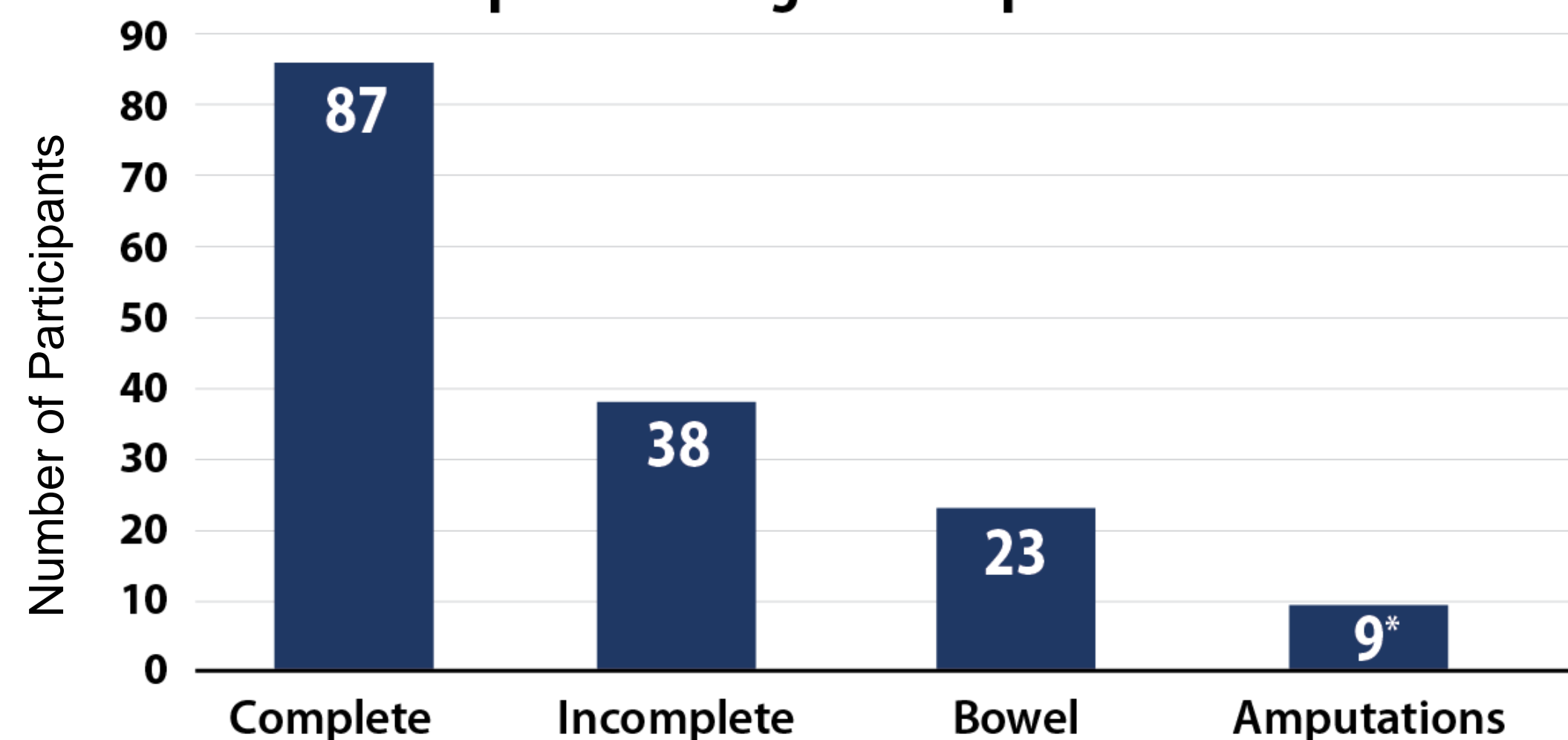
Background

- The Desmoid Tumor Research Foundation (DTRF) launched the natural history study (NHS) in 2017.
- Desmoid tumor participants reported responses to treatments by location to many treatment modalities, including active surveillance and systemic agents.

Results

- Treatment data collected from 270 participants.
- Active surveillance or no systemic treatment was reported by 47.6% participants.
- Surgery was the 1st intervention for all locations (49/81, 60%), except those with head/neck tumors who received chemotherapy (6/11, 55%).

Reported Surgical Responses



*Note: 8 reported recurrent disease following the removal of the limb

- Surgery was the 1st line of treatment for all tumor locations except head and neck.
- 8 out of 9 patients reported recurrent tumor after amputation.
- Targeted agents are used less than half as often outside of the US.
- Participants reported that targeted agents prevented tumor growth better than chemotherapy.

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THE DESMOID TUMOR RESEARCH FOUNDATION

* The authors are humbled by the ongoing contributions by the desmoid tumor patients and their caregivers and to NORD for their support of all rare disorders.

Results continued

- Active surveillance was most common for desmoid tumors located in abdominal wall (54/103; 52.4%).
- Radiation and HiFU were mostly described for participants with chest wall tumors (15 participants) and joints/extremities (10 participants).
- The use of targeted agents was greater in the US compared to ex-US countries (21% vs 9%).
- Systemic therapies prescribed to participants:
 - Sorafenib (44/284; 15.5%)
 - Sulindac (36/284; 12.7%)
 - Anti-hormonal agents tamoxifen and toremifene (34/285; 10.9%)

Participant Reported Responses to Systemic Treatments

