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Comparative Effectiveness of Systemic Treatments in Desmoid Tumors

Background: Many systemic treatment options are described for desmoid tumors, including hormonal therapies, non-steroidal anti-inflammatory drugs, cytotoxic chemotherapeutic agents, and most recently, tyrosine kinase inhibitors. Although many options are described, randomized data is scarce. The lack of comparative studies precludes a definitive sequence of the existing systemic treatments in the management of desmoid tumors. Here we retrospectively compare sorafenib with cytotoxic chemotherapy to generate objective data to guide treatment choice.

Methods: We analyzed all patients with desmoid tumors treated with doxorubicin, dacarbazine, vinblastine, vinorelbine, methotrexate, or sorafenib in the first-line setting at a single center from 2000-2021. The primary endpoint was investigator-assessed progression-free survival. The secondary endpoint was the rate of toxic effects recorded accordingly to the Common Terminology Criteria for Adverse Events. We calculated progression-free survival (PFS) using the Kaplan-Meier method with Log-Rank Test to estimate the 95% confidence interval.

Results: 79 patients ultimately received systemic therapies. Median follow-up was 5.6 years (0 to 7.9), 69% were women, and median age at diagnosis was 37 (range 5-77). The tumor was in the lower extremity in 21 (27%) cases, trunk in 18 (23%) cases, abdominal wall in 13 (16%), intra-abdomen in 9 (11%), upper extremity in 7 (9%), head-neck in 7 (9%) and breast in 4 (5%). Surgery before systemic treatment was used in 20 (25%) patients. The regiments used were sorafenib (n=32), doxorubicin with dacarbazine (n=13), liposomal doxorubicin (n=11), methotrexate with vinblastine (n=11), methotrexate with vinorelbine (n=8) and methotrexate monotherapy (n=1). The 2-year progression-free survival rate was 80% (95% confidence interval [CI], 0.64 to 0.96) in the cytotoxic chemotherapy group and 66% (95% CI, 0.46 to 0.86) in the sorafenib group (P=0.06). The most frequently reported adverse events in the cytotoxic group were grade 1 or 2 events of nausea (25%) and rash (15%), and on sorafenib were grade 1 or 2 events of palmar-plantar erythrodysesthesia (40%) and fatigue (25%). There were no treatment-related deaths reported.

Conclusion: There was no statistical difference between the three analyzed treatments, although there was a trend toward lower progression rates with cytotoxic therapy.