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Systemic treatment preferences for patients with advanced desmoid-type fibromatosis in Europe

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Background: The treatment of desmoid type fibromatosis (DF) as a rare, benign mesenchymal tumor is poorly standardized and mainly based on physician's choice. Only a few clinical trials have assessed the different treatment options in DF and randomized evidence is virtually non-existing. We evaluated systemic treatment preferences for patients with advanced DF among European sarcoma experts, aiming to provide a reference for a generally acceptable control treatment for potential prospective randomized trials.

Methods: A 7-item structured questionnaire was sent to physicians in Europe and Israel involved in the multidisciplinary care and experimental treatment of mesenchymal tumors, to assess their systemic treatment choices for DF.

Results: The questionnaire was sent to 266 experts (117 institutions, 21 countries) and 54 physicians (52 institutions, 14 countries) responded. "Wait-and-see" was the most common primary approach for patients with advanced DF. (Symptomatic) disease progression and failure or non-availability of local treatment options were cited as common reasons for considering systemic therapy among physicians. Treatment preferences for patients with sporadic vs. hereditary DF were similar, with a tendency of earlier use of chemotherapy in hereditary DF. At least 28 different agents or regimens are preferred choices in DF among the responding caregivers. Tamoxifen +/- nonsteroidal anti-inflammatory agents (NSAIDs) or NSAIDs alone are the most commonly cited preferred treatment choices in first line, followed by methotrexate- or anthracycline-containing regimen. Tyrosine kinase inhibitors are more preferred in subsequent lines of DF treatment by European physicians; other drugs are used only sporadically. Clinical trial activity in DF was restricted to only one country and one multi-centric study. Detailed results of the questionnaire assessment will be presented at the meeting.

Conclusions: DF patients in Europe and Israel seem to have broad off label access to a variety of systemic agents representing diverse drug classes and modes of action, but clinical trial options are very limited. Treatment approaches are diverse, poorly standardized and based on weak evidence. There is an urgent need for practice-defining, prospective randomized trials. Such trials should ideally select a homogeneous population of patients with

well documented, progressive, symptomatic disease and/or functional impairment after “wait-and-see” and/or local treatments. NSAIDs +/- tamoxifen could be considered a generally accepted standard control treatment for clinical trials at least among physicians in Europe.