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ABSTRACT

Purpose

To prospectively assess the behaviour of primary sporadic (not FAP associated) desmoid fibromatosis (DF) managed by active surveillance (AS).

Experimental design

This is an Italian prospective, multicenter, observational study (NCT 02547831) including patients ≥16 years with primary sporadic DF at any site.

Patients were assessed by Response Evaluation Criteria in Solid Tumor (RECIST) version 1.1. Primary end-point was progression-free survival (PFS) at 3 years. Treatment-free survival (TFS) was also analyzed. PFS and TFS were calculated by Kaplan-Meier plots and compared by log-rank test Cox proportional-hazard multivariable regression analyses were performed.

Results

From 2013 to 2018 108 consecutive patients were included (82% female); median age was 39-yr; median size was 51 mm.

CTNNB1 mutations were: T41A (50%); S45F (12%); other (19%); WT (19%).

At 32.3-month median-FU, 42/108 (39%) showed RECIST progression. Spontaneous regression (SR) was initially observed in 27/108 (25%), while it followed dimensional progression in other 33/108 (31%). PFS at 36 months was 54.5% (95% CI, 44.9%-66.1%). Thirty-five/108 (32%) patients received active treatment, 18/108 (17%) after RECIST progression and 17/108 (15%) after symptomatic progression. TFS at 36 months was 65.9% (95% CI, 57.3%-75.9%). Larger tumor size and extremity location

were associated to shorter TFS and a trend for S45F mutation was also observed (p=0.06), while none of the mentioned variables was significantly associated to PFS.

Conclusions

In primary DF, AS can be proposed, since disease stabilization and SR frequently occur. However extra care should be taken for patients with tumors of larger size, extremity location and S45F mutation.