10513 **Poster Discussion Session**

Famitinib for familial adenomatous polyposis-associated aggressive desmoid tumors: A single-center exploratory study.

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Background: Desmoid tumour (DT) is a common extra-intestinal manifestation of familial adenomatosis polyposis (FAP), with high risk of recurrence and became the second leading cause of FAP patients' death. The treatment of FAP-associated DTs are limited. Famitinib is a orally bioavailable, tyrosine kinase inhibitor against VEGFR-2, PDGFR, c-kit, and FGFR, and has shown antitumor activity in these patients (pts). **Methods:** This is a prospective, single arm study of FAP pts with progressing DT per RECIST v1.1, treated with famitinib 20 mg QD since 11/2021. The primary endpoint was objective response rate (ORR), with secondary endpoints of safety, progression-free survival (PFS), and quality of life (EORTC QLQ C30). We also collected the FFPE slices of DTs to test both genetic and proteomic backgrounds of pts with different therapeutic results. **Results:** Between Nov 24, 2021 and Aug 8, 2022, 11 pts were enrolled and evaluated every 9 weeks. The median follow-up time was 9.4 m (6.6 to 14.9 m). The median age was 36 (28-53) and 45% were male. Nine pts were diagnosed with intra-abdominal (IA) tumors while two had extra-abdominal (EA) DTs. Eight had stage III-IV DTs with rapid growth and severe symptoms. 10 of them had history of intestinal resection. Two pts were treated with dacarbazine and epirubicin before enrollment, while one patient received radiotherapy before famitinib. Five pts achieved partial response (PR) and six achieved stable disease (SD), with a median time to response of 7.0 months. The ORR was 45.5% and the disease control rate (DCR) was 100%. The 6-month PFS rate was 91%. The ORR of pts with IA DTs was 55.6%. Of adverse events (AEs) with famitinib, 87% were grade 1/2, the most frequently reported being hypertension (73%), neutropenia (64%), bilirubinemia (55%) and proteinuria (36%). Mean global health status/QoL before and after 6 months' treatment was 58.3 vs 75.8 (P= 0.09). Of all 11 pts, germline APC mutations scattered from codon 457 to 1578, and five of them detected somatic APC mutations from codon 547 to 1896 in DTs. Correlative study analysis will be presented. **Conclusions:** Famitinib demonstrated promising efficacy and a favorable safety profile in FAP-associated aggressive DTs. Research Sponsor: National Natural Science Foundation of China (No. 81902514).