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Efficacy and safety of pazopanib in the treatment of pediatric sarcoma: Retrospective cohort study.

Emel Cabi Unal, Sonay Incesoy Ozdemir, Nurdan Tacyildiz, Handan Dincaslan, Caglar Dogrul, Ezgi Guven, Melek Yaman Ortakoylu, Asli Bozer, Gulsan Yavuz; Ankara University Department of Pediatrics, Div of Ped Hematology/Oncology, Ankara, Turkey; Ankara University Faculty of Medicine, Department of Pediatrics - Division of Pediatric Hematology-Oncology, Ankara, Turkey; Department of Pediatrics, Ankara University Faculty of Medicine, Dikimevi, Turkey; Ankara University Faculty of Medicine, Ankara, Turkey; Ankara University Faculty of Medicine, Department of Pediatrics - Department of Statistics, Ankara, Turkey; Department of Pediatric Oncology, School of Medicine, Ankara University, Ankara, Turkey

Background: The presence of frequent genetic alterations activating the tyrosine kinase (TKI) signaling pathway has recently led to a more targeted therapeutic approach. We describe our institutional experience using pazopanib for pediatric sarcomas. **Methods:** We performed a retrospective, IRB approved, chart review of all patients with sarcoma treated with pazopanib off-study at Ankara University Pediatric Oncology Clinic between 2019–2021. **Results:** Twenty-five patients were identified. Median age at commencement of pazopanib treatment was 13 years old (range 4 to18 years; 15 male: 10 female). Median duration on treatment with pazopanib was 16 months (range, 2 to 33). The median duration between the diagnosis of sarcoma and the beginning of pazopanib was 17.0 months (range, 1 to 80 months). Twelve of 25 patients due to metastatic disease at diagnosis, pazopanib was started with first-line chemotherapy and/or continued as maintenance therapy (Group 1). In the remaining 13 of 25 patients, received a second-line chemotherapy with pazopanib was administrated due to relapsed disease (Group 2). Nine patients (36.0%) (2 patients in group 1 and 7 patients in group 2) had died from progressive disease ranging between 13 and 70 months (median 33 months) after the diagnosis. OS rate was 64,0% in the entire cohort; group 1 and group 2 were 83,3%, and 46,2%, respectively. The overall median OS was 69.4 months. No new pazopanib-related adverse effects were identified. **Conclusions:** Pazopanib appears to be an effective treatment for pediatric sarcoma. Treatment with pazopanib is well-tolerated and could have a role to maintain response in patients with pediatric sarcoma. Further studies are needed to better define the use of this regimen in the upfront management of those patients. Research Sponsor: None.

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