Clinical Trial JCO Precis Oncol. 2024 Apr:8:e2300407. doi: 10.1200/PO.23.00407.

Phase II Study of Erdafitinib in Patients With Tumors With Fibroblast Growth Factor Receptor Mutations or Fusions: Results From the NCI-MATCH ECOG-ACRIN Trial (EAY131) Subprotocol K2

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PMID: 38603650 DOI: 10.1200/PO.23.00407

Abstract

Purpose: Subprotocol K2 (EAY131-K2) of the NCI-MATCH platform trial was an open-label, single-arm, phase II study designed to evaluate the antitumor efficacy of the oral FGFR1-4 inhibitor, erdafitinib, in patients with tumors harboring FGFR1-4 mutations or fusions.

Methods: Central confirmation of tumor FGFR1-4 mutations or fusions was required for outcome analysis. Patients with urothelial carcinoma were excluded. Enrolled subjects received oral erdafitinib at a starting dose of 8 mg daily continuously until intolerable toxicity or disease progression. The primary end point was objective response rate (ORR) with key secondary end points of safety, progression-free survival (PFS), and overall survival (OS).

Results: Thirty-five patients were enrolled, and 25 patients were included in the primary efficacy analysis as prespecified in the protocol. The median age was 61 years, and 52% of subjects had received ≥3 previous lines of therapy. The confirmed ORR was 16% (4 of 25 [90% CI, 5.7 to 33.0], P = .034 against the null rate of 5%). An additional seven patients experienced stable disease as best-confirmed response. Four patients had a prolonged PFS including two with recurrent WHO grade IV, IDH1-/2-wildtype glioblastoma. The median PFS and OS were 3.6 months and 11.0 months, respectively. Erdafitinib was manageable with no new safety signals.

Conclusion: This study met its primary end point in patients with several pretreated solid tumor types harboring FGFR1-3 mutations or fusions. These findings support advancement of erdafitinib for patients with fibroblast growth factor receptor-altered tumors outside of currently approved indications in a potentially tumor-agnostic manner.